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TITLE: Understanding and Targeting Epigenetic Alterations in Acquired Bone Marrow Failure

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14. ABSTRACT

Systematic genomic discovery efforts in patients with bone marrow failure due to myelodysplastic syndrome (MDS) has led to the rapid discovery of recurrent somatic genetic alterations underlying these disorders. Remarkably, a large number of these mutations occur in genes whose function is known, or suspected, to be involved in epigenetic regulation of gene transcription or in RNA splicing. This includes mutations in *ASXL1, TET2,* and *EZH2* as well as mutations in the RNA splicing factors SF3B1, SRSF2, and U2AF1. Over the course of funding of this award we have made major progress in (1) understanding the impact of *ASXL1* mutations and loss on chromatin (Abdel-Wahab, *et al. Cancer Cell* 2012), (2) identifying the *in vivo* biological effects of deletion of *Asxl1* and *Tet2* alone and in combination with one another (Abdel-Wahab, *et al. J Exp Med* 2013), (3) identified the genome-wide effects of Asxl1 on transcription (Abdel-Wahab, *et al. J Exp Med* 2013 and Abdel-Wahab, O*, et al. Leukemia* 2013), (4) identified that mutations in the splicing machinery in MDS also may impact the function of epigenetic modifiers (Kim, E, *et al. Cancer Cell* 2015), (5) developed therapeutic approach to target spliceosomal mutant MDS (Lee, SCW, *et al. Nat Med* 2016), and (6) identified a function of ASXL2, a paralog of ASXL1, in normal and malignant hematopoiesis (Micol, J-B, *et al. Nat Comm* 2017).

15. SUBJECT TERMS
ASXL1; Bone marrow failure; Myelodysplastic Syndrome; RNA splicing; SF3B1; SRSF2; TET2

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Introduction

Increasing use of genomic discovery efforts in patients with bone marrow failure due to myelodysplastic syndrome (MDS) has led to the rapid discovery of a series of recurrent genetic abnormalities underlying these disorders. Remarkably, a large number of these alterations appear to be in genes whose function is known, or suspected, to be involved in (1) epigenetic regulation of gene transcription and (2) mRNA splicing. This includes mutations in the genes encoding the epigenetic modifiers TET2, ASXL1, DNMT3a, and EZH2 have all been found to be frequent mutations amongst patients with MDS. In addition, mutations in the spliceosomal genes *SRSF2*, *U2AF1* and *SF3B1* are now known to be commonly found in patients with MDS. These mutations occur at highly restricted amino acid residues, are always heterozygous, and never co-occur with one another. These data suggest that splicing mutations confer an alteration of splicing function and/or that cells may only tolerate a certain degree of splicing modulation.

Identification of frequent mutations in epigenetic modifiers and RNA splicing factors has highlighted the fact that a number of these genes encode enzymes and/or result in alterations in enzymatic function which may represent novel, tractable therapeutic targets for MDS patients. In this proposal, we originally aimed to identify (a) if mice with genetically engineered deletion of epigenetic modifiers mutated in MDS would serve as valuable murine models of MDS, (b) if mutations in epigenetic modifiers may specifically impact DNA methylation and/or histone post-translational modifications in a manner that is therapeutically targetable, and (c) if additional mutations must exist in patients with specific subsets of MDS with the worst clinical outcome. Since awarding of the proposal, we have made major insights into the epigenomic function of ASXL1 as well as the biological impact of conditional deletion of *Asxl1* alone and in combination with other genetic alterations including *Tet2* deletions and NRasG12D overexpression. In addition, we have recently identified that an additional class of very frequency mutations in MDS patients affecting the spliceosome impacts EZH2 function.

We previously showed that mice expressing the heterozygous *Srsf2P95H* mutation develop MDS-like features due to altered RNA binding and splicing preference of the mutant protein. These biological and mechanistic features of the mutant SRSF2 protein are distinct from those seen with loss of 1 or copies of SRSF2, indicating that SRSF2 mutations confer an alteration of function. Specifically, mutations in SRSF2 alter its binding to exonic splicing enhancers (ESEs) such that the mutant protein recognizes C-rich ESE sequences over G-rich ESEs whereas the wildtype protein recognizes C- and G-rich ESEs similarly. Recent work from others has revealed that mutations in the core spliceosomal protein U2AF1 also result in altered RNA biding and splicing preference based on the nucleotide sequence immediately surrounding the 3' splice site. Finally we have identified that spliceosomal mutant MDS cells display greater sensitivity towards pharmacologic inhibition of splicing function than spliceosomal wildtype counterparts. This latter finding has resulted in a novel phase I clinical trial of spliceosome inhibitor compound in patients with refractory MDS and other myeloid leuekmias (clinicaltrials.gov identifier NCT02841540). In addition, this work has resulted in several publications, multiple oral presentations at national meetings, and has been used as the basis for several additional foundation and NIH R01 awards.

Keywords:

5-azacytidine, ASXL1, Decitabine, Epigenetics, EZH2, Genomics, Mouse models, Myelodysplastic Syndromes, Splicing, SF3B1, SRSF2, TET2.

Accomplishments

Key Research Accomplishments

- Developed and published the first conditional knockout mouse for *Asxl1* as well as the first murine model with combined *Asxl1* and *Tet2* deletion. We believe these models are valuable genetically accurate murine models of acquired bone marrow failure.
- Identified the biological effects of *Asxl1* loss on hematopoiesis, alone and in combination with other co-occurring genetic alterations.
- Generated the first murine model of spliceosomal mutations as seen in patients with MDS.
- Identified an important intersection of spliceosomal gene alterations on the epigenome of MDS.
- Identified a novel therapeutic approach for cells bearing spliceosomal gene alterations.
- Identified the biological role for ASXL2, a paralog of ASXL1, in normal and malignant hematopoiesis.

In addition to the above summary, below is a more detailed summary of accomplishments organized by Tasks from the original grant submission:

Task 1. "Obtain DoD ACURO approval for the use of animals in the experiments outlined below in Tasks 2 to 4."

This was completed.

Task 2. "Complete characterization of mice with conditional deletion of Asxl1 alone and Asxl1 combined with Tet2 (Months 1-24) at the work performance site of Memorial Sloan-Kettering Cancer Center."

This work was published in 2013 in the *Journal of Experimental Medicine* (**Abdel-Wahab, O**, *et al*. *J Exp Med* 2013 Nov 18;210(12):2641-59) and have been used by the MDS research community internationally. We have deposited these mice at the Jackson Laboratory for public use.

In addition, we also recently created mice with compound loss of Asxl1 and Asxl2 in order to understand the role and potential redundancy of Asxl2 with Asxl1 in hematopoiesis. This work is now in press at *Nature Communications* in the following publication:

Micol, JB, Pastore, A, Inoue, D, Duployez, N, Kim, E, Lee, SCW, Durham, BH, Chung, YR, Cho,H, Zhang, XJ, Yoshimi, A, Krivtsov, A, Koche, R, Solary, E, Sinha, A, Preudhomme, C, **Abdel-Wahab, O**. ASXL2 is essential for normal hematopoiesis and a haploinsufficient tumor suppressor in leukemia. *Nature Communications* 2017 (In press)

Task 3. Continue development of mice with Ezh2 deletion alone and characterize mice with compound deletion of Ezh2/Tet2 and Ezh2/Asxl1 (Months 1-24) at the work performance site of Memorial Sloan-Kettering Cancer Center.

We recently generated mice with Ezh2 deletion in the postnatal compartment (*Mx1-cre Ezh2fl/fl*) mice and mice with compound deletion of *Ezh2* and *Asxl1.* From these murine models we have identified that:

(i) Hematopoietic stem cells (HSCs) from mice with compound *Asxl1/Ezh2* loss have impaired self-renewal compared with HSCs from littermate control mice as well as mice with deletion of either gene alone.

(ii) A high proportion of wildtype mice reconstituted with bone marrow from mice with compound *Asxl1/Ezh2* (Mx1-cre Asxl1fl/fl Ezh2fl/fl) deletion die of bone marrow failure within weeks of deletion of these genes. Surviving mice are characterized by anemia and leukopenia as well as morphologic dysplasia.

The above phenotypes of mice with compound deletion of both Asxl1 and Ezh2 are dramatic and we are now working to functionally understand the mechanism by which deletion of these 2 genes impairs HSC function.

In addition to the above, we have recently identified the unexpected observation that mutations in the spliceosomal protein SRSF2, commonly identified in MDS patients, results in mis-splicing of *EZH2*. Interestingly, *SRSF2* mutations and loss-of-function EZH2 mutations in MDS are 100% mutually exclusive but the functional basis for this interaction was not known previously. Our work provided the basis for this observation and identified another mechanism by which EZH2 is dysregulated in MDS. These data were published in the following manuscript:

Kim E, Ilagan JO, Liang Y, Daubner GM, Lee SC, Ramakrishnan A, Li Y, Chung YR, Micol JB, Murphy ME, Cho H, Kim MK, Zebari AS, Aumann S, Park CY, Buonamici S, Smith PG, Deeg HJ, Lobry C, Aifantis I, Modis Y, Allain FH, Halene S, Bradley RK, **Abdel-Wahab O**. SRSF2 Mutations Contribute to Myelodysplasia by Mutant-Specific Effects on Exon Recognition. Cancer Cell. 2015 May 11;27(5):617-30. doi: 10.1016/j.ccell.2015.04.006. PubMed PMID: 25965569; PubMed Central PMCID: PMC4429920.

Task 4. Determine the epigenetic contribution of Asxl1 and Ezh2 loss to bone marrow failure through Chromatin immunoprecipitation (ChIP) of histone H3 lysine 27 trimethyl (H3K27me3) followed by next-generation sequencing in primary murine hematopoietic cells (Months 1-24) at the work performance site of Memorial Sloan-Kettering Cancer Center.

As noted in 2 prior annual reports, we have completed detailed characterization of the effects of *ASXL1* mutations and loss using cell lines and primary cells from knockout mice. These results have been published now in 2 papers (Abdel-Wahab, O, *et al. Cancer Cell* 2012 and Abdel-Wahab, O, *et al. J Exp Med* 2013).

Task 5: Determine the effect of Tet2, Asxl1, and Ezh2 loss to a panel of currently clinically utilized compounds in patients with MDS. Drug panel will include decitabine, 5-azacytidine, lenalidomide, cytarabine, daunorubicin, HDACi (vorinostat, romidepsin, panobinostat, AR-42, trichostatin A), HSP-90 inhibitors (AUY-922, PUH-71), and parthenolide (Months 1-24) at the work performance site of Memorial Sloan-Kettering Cancer Center.

We are now performing these experiments *ex vivo* through use of methylcellulose colony assays. In brief, hematopoietic stem/progenitor cells (HSPCs; lineage-negative Sca1+ c-KIT+ cells) from Tet2 knockout, Asxl1 knockout, Ezh2 knockout, and Tet2/Asxl1 double knockout mice are being plated in methylcellulose with a variety of the above compounds for 7 days. We are evaluating the effects of these compounds on restoring colony formation (for Asxl1 and Ezh2 knockout HSPCs) or reducing colony formation (for Tet2 and Tet2/Asxl1 knockout HSPCs). This work is underway.

In addition to the above experiments, we have identified that spliceosomal mutant MDS and other cancer cells are critically dependent on wildtype splicing catalysis. This was identified in a paper we published in *Nature Medicine* last year and an ongoing phase I clinical trial (clinicaltrials.gov identifier NCT02841540):

Lee SC, Dvinge H, Kim E, Cho H, Micol JB, Chung YR, Durham BH, Yoshimi A, Kim YJ, Thomas M, Lobry C, Chen CW, Pastore A, Taylor J, Wang X, Krivtsov A, Armstrong SA, Palacino J, Buonamici S, Smith PG, Bradley RK, **Abdel-Wahab O**. Modulation of splicing catalysis for therapeutic targeting of leukemia with mutations in genes encoding spliceosomal proteins. *Nat Med*. 2016 Jun;22(6):672-8. doi: 10.1038/nm.4097. Epub 2016 May 2. PubMed PMID: 27135740; PubMed Central PMCID: PMC4899191.

Task 5: Perform candidate gene and exome sequencing on DNA samples from 20 MDS patients with *ASXL1* mutations alone (Months 1-6) at the work performance site of Memorial Sloan-Kettering Cancer Center.

In order to complete this task and to inform task #5, we recently performed targeted DNA sequencing on pretreatment DNA samples from a cohort of MDS patients uniformly treated with decitabine. This work, performed in collaboration with MDS clinical expert Dr. Valeria Santini, revealed that *ASXL1* mutations frequently co-occur with mutations in the spliceosome-associated protein *SRSF2* in patients with MDS/MPN overlap syndromes. This interesting finding suggests an interaction by mutations in the epigenome with mutations in the spliceosome. Moreover, this work has resulted in one recent publication as noted above (in “Task 5”).

Task 6: Perform candidate gene and exome sequencing on DNA samples from 40 patients with MDS accompanied by moderate to severe bone marrow fibrosis (Months 1-6) at the work performance site of Memorial Sloan-Kettering Cancer Center.

We have now collected samples from 40 such patients with MDS with bone marrow fibrosis and hope to begin performing DNA sequencing soon. We recently helped to generate a DNA next-generation sequencing panel of 300 genes implicated in cancer pathogenesis at our institution. We will apply this sequencing platform to these MDS samples with the hopes of characterizing any novel mutations associated with this unique subtype of MDS.

Task 7: Present findings at national meetings and publish in peer-reviewed journals (Month 6-36).

I have given >20 presentations at national/international meetings on the work performed with funding from this award in the last year (see list of presentations in **Products** below).

I have also been invited to write several reviews related to the work described in this proposal in well-respected journals including *Nature Medicine, Genes & Development, Blood,* and *Nature Reviews Cancer* (cited in **Products** below).

Impact

Genomic discovery efforts in patients with MDS have revealed that the most frequent somatic mutations in these disorders are in genes involved in either epigenetic regulation or RNA splicing. We and others have recently shown that mutations in the Polycomb-associated gene *ASXL1* and the spliceosmal gene *SRSF2* have adverse prognostic importance in patients with all myeloid malignancies including MDS, acute myeloid leukemia (AML), chronic myelomoncytic leukemia (CMML), and primary myelofibrosis. We therefore have focused on understanding the role of these mutations in MDS pathogenesis. In brief, we have identified that the loss-of-function mutations in *ASXL1* as well as the gain-of-function mutations in *SRSF2* both converge on decreased function of the Polycomb Repressive Complex 2 (PRC2). This work has resulted in multiple genetically accurate models of MDS as well as reagents to screen for novel therapeutic targets for *TET2-, ASXL1-* or *SRSF2*-mutant cells.

Changes/Problems

Nothing to report.

Reportable Outcomes

Original Manuscripts:

1. Kim, E, Ilagan, JO (co-first author), Liang, Y (co-first author), Daubner, GM (co-first author), Lee, S, Ramakrishnan, A, Li, Y, Chung, YR, Micol, J-B, Murphy, M, Cho, H, Kim, M-K, Zebari, AS, Buonamici, S, Smith, P, Deeg, HJ, Lobry, C, Aifantis, I, Modis, Y, Allain, F.H.-T., Halene, S (co-corresponding), Bradley, RK (co-corresponding), **Abdel-Wahab, O (co-corresponding)**. SRSF2 Mutations Contribute to Myelodysplasia Through Mutant-Specific Effects on Exon Recognition. *Cancer Cell*. 2015 May 11;27(5):617-30. doi: 10.1016/j.ccell.2015.04.006. PubMed PMID: 25965569; PubMed Central PMCID: PMC4429920.
2. Fong CY, Gilan O, Lam EY, Rubin AF, Ftouni S, Tyler D, Stanley K, Sinha D, Yeh P, Morison J, Giotopoulos G, Lugo D, Jeffrey P, Lee SC, Carpenter C, Gregory R, Ramsay RG, Lane SW, **Abdel-Wahab O**, Kouzarides T, Johnstone RW, Dawson SJ, Huntly BJ, Prinjha RK, Papenfuss AT, Dawson MA. BET inhibitor resistance emerges from leukaemia stem cells. *Nature*. 2015 Sep 24;525(7570):538-42. doi: 10.1038/nature14888. Epub 2015 Sep 14. PubMed PMID: 26367796.
3. LaFave LM, Béguelin W, Koche R, Teater M, Spitzer B, Chramiec A, Papalexi E, Keller MD, Hricik T, Konstantinoff K, Micol JB, Durham B, Knutson SK, Campbell JE, Blum G, Shi X, Doud EH, Krivtsov AV, Chung YR, Khodos I, de Stanchina E, Ouerfelli O, Adusumilli PS, Thomas PM, Kelleher NL, Luo M, Keilhack H, **Abdel-Wahab O,** Melnick A, Armstrong SA, Levine RL. Loss of BAP1 function leads to EZH2-dependent transformation. *Nat Med.* 2015 Nov;21(11):1344-9. doi: 10.1038/nm.3947. Epub 2015 Oct 5. PubMed PMID: 26437366; PubMed Central PMCID: PMC4636469.

4. Zhang L, Tran NT, Su H, Wang R, Lu Y, Tang H, Aoyagi S, Guo A, Khodadadi-Jamayran A, Zhou D, Qian K, Hricik T, Côté J, Han X, Zhou W, Laha S, **Abdel-Wahab O,** Levine RL, Raffel G, Liu Y, Chen D, Li H, Townes T, Wang H, Deng H, Zheng YG, Leslie C, Luo M, Zhao X. Cross-talk between PRMT1-mediated methylation and ubiquitylation on RBM15 controls RNA splicing. *Elife*. 2015 Nov 17;4. pii: e07938. doi: 10.7554/eLife.07938. [Epub ahead of print] PubMed PMID: 26575292.
5. Guryanova, O, Lieu, Y, Garrett-Bakelman, FE, Spitzer, B, Glass, J, Shank, K, Valencia Martinez, AB, Rivera, S, Durham, B, Rapaport, F, Keller, M, Pandey, S, Bastian, L, Tovbin, D, Weinstein, A, Teruya-Feldstein, J, **Abdel-Wahab, O**, Santini, V, Mason, C, Melnick, A, Mukherjee, S, Levine, RL. Dnmt3a Regulates Myeloproliferation and Liver-Specific Expansion of Hematopoietic Stem and Progenitor Cells. *Leukemia*. 2016 May;30(5):1133-42. doi: 10.1038/leu.2015.358. PubMed PMID: 26710888; PubMed Central PMCID: PMC4856586.
6. Taggart J, Ho TC, Amin E, Xu H, Barlowe TS, Perez AR, Durham BH, Tivnan P, Okabe R, Chow A, Vu L, Park SM, Prieto C, Famulare C, Patel M, Lengner CJ, Verma A, Roboz G, Guzman M, Klimek VM, **Abdel-Wahab O**, Leslie C, Nimer SD, Kharas MG. MSI2 is required for maintaining activated myelodysplastic syndrome stem cells. *Nat Commun*. 2016 Feb 22;7:10739. doi: 10.1038/ncomms10739. PubMed PMID: 26898884; PubMed Central PMCID: PMC4764878.
7. Lee SC, Dvinge H, Kim E, Cho H, Micol JB, Chung YR, Durham BH, Yoshimi A, Kim YJ, Thomas M, Lobry C, Chen CW, Pastore A, Taylor J, Wang X, Krivtsov A, Armstrong SA, Palacino J, Buonamici S, Smith PG, Bradley RK, **Abdel-Wahab O**. Modulation of splicing catalysis for therapeutic targeting of leukemia with mutations in genes encoding spliceosomal proteins. *Nat Med*. 2016 Jun;22(6):672-8. doi: 10.1038/nm.4097. Epub 2016 May 2. PubMed PMID: 27135740; PubMed Central PMCID: PMC4899191.
8. Zhang, P, Xing, C, Rhodes, SD, He, Y, Deng, K, Li, Z, He, F, Zhu, C, Nguyen, L, Zhou, Y, Chen, S, Mohammad, KS, Guise, TA, **Abdel-Wahab, O,** Xu, M, Wang, QF, Yang, FC. Loss of Asxl1 Alters Self-Renewal and Cell Fate of Bone Marrow Stromal Cell, Leading to Bohring-Opitz-like Syndrome in Mice. *Stem Cell Reports*. 2016 May 24. pii: S2213-6711(16)30038-8. doi: 10.1016/j.stemcr.2016.04.013. [Epub ahead of print] PubMed PMID: 27237378
9. Lu R, Wang P, Parton T, Zhou Y, Chrysovergis K, Rockowitz S, Chen WY, **Abdel-Wahab O,** Wade PA, Zheng D, Wang GG. Epigenetic Perturbations by Arg882-Mutated DNMT3A Potentiate Aberrant Stem Cell Gene-Expression Program and Acute Leukemia Development. *Cancer Cell.* 2016 Jul 11;30(1):92-107. doi: 10.1016/j.ccell.2016.05.008. Epub 2016 Jun 23. PubMed PMID: 27344947; PubMed Central PMCID: PMC4945461.
10. Shi H, Yamamoto S, Sheng M, Bai J, Zhang P, Chen R, Chen S, Shi L, **Abdel-Wahab O,** Xu M, Zhou Y, Yang FC. ASXL1 plays an important role in erythropoiesis. *Sci Rep*. 2016 Jun 29;6:28789. doi: 10.1038/srep28789. PubMed PMID: 27352931; PubMed Central PMCID: PMC4926121.
11. Guryanova, O, Shank, K, Spitzer, B, Luciani, L, Koche, R, Garrett-Bakelman, F, Ganzel, C, Durham, B, Mohanty, A, Hoermann, G, Rivera, S, Chramiec, A, Pronier, E, Bastian, L, Keller, M, Tovbin, D, Loizou, E, Weinstein, A, Rodriguez Gonzalez, A, Lieu, Y, Rowe, J, Pastore, F, McKenney, A, Krivtsov, A, Sperr, WR, Cross, J, Mason, C, Tallman, M, Arcila, ME, **Abdel-Wahab, O,** Armstrong, S, Kubicek, S, Staber, P, Gönen, M, Paietta, E, Melnick, A, Nimer, S, Mukherjee, S, Levine, RL. DNMT3A R882 mutations promote anthracycline resistance in acute

myeloid leukemia through impaired nucleosome remodeling. *Nat Med.* 2016 Dec;22(12):1488-1495. doi: 10.1038/nm.4210. Epub 2016 Nov 14. PubMed PMID: 27841873; PubMed Central PMCID: PMC5359771.

12. Micol, JB, Pastore, A, Inoue, D, Duployez, N, Kim, E, Lee, SCW, Durham, BH, Chung, YR, Cho,H, Zhang, XJ, Yoshimi, A, Krivtsov, A, Koche, R, Solary, E, Sinha, A, Preudhomme, C, **Abdel-Wahab, O**. ASXL2 is essential for normal hematopoiesis and a haploinsufficient tumor suppressor in leukemia. *Nature Communications* 2017 (In press)
13. Jin, S, Su, H, Tran, N-T, Song, J, Li, Y, Lu, SX, Huang, S, **Abdel-Wahab, O,** Liu, Y, Zhao, X. SF3B1K700E mutant dysregulates erythroid differentiation via aberrant alternative splicing of transcription factor TAL1. *PLoSOne* 2017 (in press)

Review Papers:

1. Inoue D, Bradley RK, **Abdel-Wahab O.** Spliceosomal gene mutations in myelodysplasia: molecular links to clonal abnormalities of hematopoiesis. *Genes Dev*. 2016 May 1;30(9):989-1001. doi: 10.1101/gad.278424.116. Review. PubMed PMID: 27151974; PubMed Central PMCID: PMC4863743.
2. Dvinge H, Kim E, **Abdel-Wahab O (co-corresponding)**, Bradley RK. RNA splicing factors as oncoproteins and tumour suppressors. *Nat Rev Cancer.* 2016 Jun 10. doi: 10.1038/nrc.2016.51. [Epub ahead of print] PubMed PMID: 27282250.
3. Ntziachristos P, **Abdel-Wahab O**, Aifantis I. Emerging concepts of epigenetic dysregulation in hematological malignancies. *Nat Immunol*. 2016 Aug 1. doi: 10.1038/ni.3517. [Epub ahead of print] Review. PubMed PMID: 27478938.
4. Micol JB, **Abdel-Wahab, O**. The Role of Additional Sex Combs-Like Proteins in Cancer. *Cold Spring Harb Perspect Med*. 2016 Aug 15. pii: a026526. doi: 10.1101/cshperspect.a026526. [Epub ahead of print] PubMed PMID: 27527698.
5. Lee SC, **Abdel-Wahab, O.** Therapeutic targeting of splicing in cancer. *Nat Med.* 2016 Sep 7;22(9):976-86. doi: 10.1038/nm.4165. Review. PubMed PMID: 27603132.
6. Yoshimi A, **Abdel-Wahab, O.** Defining risk in MDS over time. *Blood*. 2016 Aug 18;128(7):885-6. doi: 10.1182/blood-2016-07-724930. PubMed PMID: 27539994.
7. Inoue, D, **Abdel-Wahab, O**. Modeling SF3B1 Mutations in Cancer: Advances, Challenges, and Opportunities. *Cancer Cell*. 2016 Sep 12;30(3):371-3. doi: 10.1016/j.ccell.2016.08.013. PubMed PMID: 27622329.
8. Yoshimi A, **Abdel-Wahab O.** Molecular Pathways: Understanding and Targeting Mutant Spliceosomal Proteins. *Clin Cancer Res.* 2017 Jan 15;23(2):336-341. doi: 10.1158/1078-0432.CCR-16-0131. PubMed PMID: 27836865; PubMed Central PMCID: PMC5241248.
9. Liu, B and **Abdel-Wahab, O.** Partial loss of genes might open therapeutic window. ELife 2017 Mar 17;6. pii: e25996. doi: 10.7554/eLife.25996. PubMed PMID: 28304277; PubMed Central PMCID: PMC5357136.
10. Joshi, P, Halene, S, **Abdel-Wahab, O**. How do mRNA splicing alterations drive myelodysplasia? *Blood* 2017 Mar 27. pii: blood-2017-02-692715. doi: 10.1182/blood-2017-02-692715. [Epub ahead of print] PubMed PMID: 28348147.

Presentations:

2016 Human Biology Seminar Series, Fred Hutchinson Cancer Research Center, Seattle, WA
2016 5th Annual Symposium of the Critical Reviews in Hematological Malignancies, King Faisal Specialist Hospital and Research Centre, Riyadh, Saudi Arabia
2016 5th International Bone Marrow Failure Disease Scientific Symposium, Aplastic Anemia and MDS International Foundation, Rockville, MD
2016 36th Annual Congress of the French Society of Hematology, Paris, France
2016 Leukemia Grand Rounds, MD Anderson Cancer Center, Houston, TX
2016 AACR Educational Session, AACR Annual Meeting, New Orleans, LA
2016 AACR Recent Advances in Diagnosis and Therapy Session, AACR Annual Meeting, New Orleans, LA
2016 Starr Cancer Consortium Retreat, Cold Spring Harbor Laboratories, Cold Spring Harbor, NY
2016 Hematology Grand Rounds, Fred Hutchinson Cancer Research Center, Seattle, WA
2016 Nature Medicine/Nature Biotechnology SciCafe "Targeting Inhibition of Splicing", New York, NY, USA
2016 1st Dotan International Symposium, Tel Aviv University, Tel Aviv, Israel
2016 Winthrop Rockefeller Seminar Series, University of Arkansas Medical School, Little Rock, Arkansas, USA
2016 Plenary Speaker, Japanese Society of Hematology, 78th Annual Meeting, Yokohama, Japan
2016 Plenary Speaker, 2nd Spliceosomal Gene Mutations in Cancer Workshop, Broad Institute of Harvard & MIT, Cambridge, MA
2016 Developmental Therapeutics Research Seminar, Amgen Inc., Thousand Oaks, CA
2016 Grand Rounds, Winshop Cancer Institute of Emory University
2016 Ingrim Cancer Center Seminar Series, Vanderbilt University School of Medicine, Nashville, TN
2016 Tisch Cancer Institute Seminar Series, Icahn School of Medicine at Mount Sinai, New York, NY
2016 11th CML & MPN Post-ASH Workshop, La Jolla, California
2016 8th Clinical Translation of Epigenetics and Cancer Therapy, Jekyll Island, GA
2017 Phase Separation and RNA Processing as Drivers of Cancer and Neurodegenerative Disease, UCSD, San Diego, CA
2017 Keystone Symposium RNA Processing in Human Disease, Taos, New Mexico
2017 EHA/ASH Translational Research Training In Hematology Course, Milan, Italy
2017 Curie Institute, Future of Oncology Symposium, Paris, France
2017 Massachusetts General Hospital Cancer Center Seminar Series, Boston, MA

Informatics:
We have generated and published multiple new mRNA sequencing (RNA-Seq) datasets as follows:

- Deep RNA-seq analysis of primary MDS patient samples with and without spliceosomal gene mutations for the purpose of identifying novel splice isoforms.
- Deep RNA-seq analysis of cells with and without spliceosomal gene mutations and with and without treatment with spliceosomal inhibitory compounds. The purpose of this dataset is to identify the effects of spliceosomal modulatory compounds on splicing and gene expression.

Funding applied for based on this work:
Applied for and successfully received numerous foundation awards and an NIH R01 award as follows:

National Institutes of Health 7/1/2015 - 6/30/2020
NIH, 1R01 HL128239 (PIs: Bradley / Abdel-Wahab)
“Genetic and molecular basis for SRSF2 mutations in myelodysplasia”

Dept. of Defense, Bone Marrow Failure Research Program 4/1/2016-3/31/2018
BM150092 (PI: Abdel-Wahab)
“Therapeutic targeting of spliceosomal mutant acquired bone marrow failure disorders”

Starr Cancer Consortium 1/1/2015 - 12/31/2016
I8-A8-075 (PI: Abdel-Wahab)
“Understanding and Targeting Spliceosomal-Mutation Hematopoietic Malignancies”

Pershing Square Sohn Cancer Research Alliance 7/1/2016 - 6/30/2019
“Identification of novel transcripts, pathways, and therapeutic strategies to target spliceosomal-mutant malignancies”

GC228160 (PI: Abdel-Wahab) 9/1/2016 - 8/31/2017
Edward P. Evans Foundation
"Elucidating Critical Targets, Transcripts, and Collaborating Events in Spliceosomal-Mutant MDS"

Conclusion

Bone marrow failure due to myelodysplastic syndrome (MDS) is driven by alterations in transcriptional regulation due to mutations in epigenetic modifiers and RNA splicing factors. Over the course of funding of this award we have made major progress in understanding the impact of mutations in both categories of alterations in MDS. This has led to the development of numerous novel genetically engineered mouse models of MDS all of which have been deposited for public use. In addition, we have developed a novel therapeutic approach to target spliceosomal mutant MDS which is currently the basis of a phase I clinical trial for patients with MDS and other refractory myeloid leukemias.

References

All papers have been cited above.

Appendices

Updated CV for Omar Abdel-Wahab

Curriculum Vitae

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Assistant Attending, Leukemia Service
Assistant Member, Human Oncology and Pathogenesis Program
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Education and Training

2007-2009	Memorial Sloan Kettering Cancer Center, New York, NY. Fellow, Hematology/Oncology.
2004-2007	Massachusetts General Hospital, Boston, MA Intern/Resident in Internal Medicine,
2000-2004	Duke University School of Medicine, M.D., *Alpha Omega Alpha*
1996-2000	Duke University, Durham, N.C. B.Sc. Biology, *Summa Cum Laude*

Research Fellowships:

2008-2011	Postdoctoral Research Fellow Human Oncology & Pathogenesis Program, Memorial Sloan-Kettering Cancer Center Ross L. Levine, MD, Associate Member
2002-2003	Medical Student Research Fellowship Dept. of Surgery, Duke University School of Medicine Doug Tyler, MD, Professor and Vice Chair Dept. of Surgery

Positions and Employment:

2016 – Current	Co-director, Hematology/Medical Oncology Fellowship Program, Dept. of Medicine, Memorial Sloan Kettering Cancer Center
2012 – Current	Assistant Level I, tenure track, Dept. of Medicine, Leukemia Service Assistant Member, Human Oncology and Pathogenesis Program Memorial Sloan-Kettering Cancer Center
2011- 2012	Assistant Level I, non-tenure track, Dept. of Medicine, Leukemia Service Memorial Sloan-Kettering Cancer Center
2010-2011	Instructor, Dept. of Medicine Memorial Sloan-Kettering Cancer Center

Honors and Awards:

2017	The Donald Seldin-Holly Smith Award for Pioneering Research, American Society of Clinical Investigation
2016	Pershing Square Sohn Prize for Young Investigators in Cancer Research

2015	Joanne Levy, MD, Memorial Award for Outstanding Achievement, American Society of Hematology (ASH)
2015	Leukemia and Lymphoma Society Clinical Scholar Award
2015	House-staff Teaching Award, MSKCC
2015	Boyer Clinical Investigator Award, MSKCC
2015	American Society of Clinical Investigation (ASCI) Young Physician-Scientist Award
2014	V Foundation Scholar Award
2013	American Society of Hematology (ASH) Junior Faculty Scholar Award
2013	Damon Runyon Clinical Investigator Award
2012	Dept. of Defense Post-doctoral Award in Bone Marrow Failure Research
2012	Josie Robertson Young Investigator Award
2012	Paul Sherlock House-staff Teaching Award, MSKCC
2011	Gabrielle's Angel Foundation Fellow Scholar Award
2010-2012	American Society of Hematology (ASH) Fellow Scholar Award
2009	American Society of Hematology (ASH) Research Training Award for Fellows
2008	Chief Fellow, Memorial Sloan Kettering Cancer Center, Medical Oncology/Hematology
2008-2010	Dana Foundation Research Fellowship
2008	John Mendelsohn House-staff Teaching Award
2004	Phillips Medical Systems Award
2004	Alpha Omega Alpha, Duke University School of Medicine
2003	Duke University School of Medicine Barham Merit Scholarship
2002	Duke University Medical Research Scholarship in General and Cardiothoracic Surgery.
1999	Phi Beta Kappa, Duke University

Licensure and Board Certification:

2007	Certification, Internal Medicine (American Board of Internal Medicine)
2007	Medical License, State of New York, #243567-1
2010	Certification, Medical Oncology (American Board of Internal Medicine)

Professional Societies:

2007	Member, American Society of Hematology
2013	Member, American Association of Cancer Research
2016	Medical Advisory Board, Bohring Opitz Syndrome Foundation

Editorial Board:

Editorial Board of *Blood* and *Haematologica*.
Associate Editor, *Clinical Cancer Reseach, International Journal of Hematology,* and *Leukemia.*

***Ad Hoc* Reviewer:**

Blood, Cancer Cell, Cancer Discovery, Cancer Research, Cell Reports, Cell Stem Cell, Clinical Cancer Research, eLIFE, Journal of Clinical Investigation, Journal of Clinical Oncology, Journal of Experimental Medicine, Leukemia, Nature, Nature Biotechnology, Nature Cell Biology, Nature Communications, Nature Genetics, Nature Medicine, Nature Reviews Cancer, New England Journal of Medicine, Oncogene, PNAS, Science, Science Immunology, Science Translational Medicine.

Publications

1. Jin, S, Su, H, Tran, N-T, Song, J, Li, Y, Lu, SX, Huang, S, **Abdel-Wahab, O**, Liu, Y, Zhao, X. SF3B1K700E mutant dysregulates erythroid differentiation via aberrant alternative splicing of transcription factor TAL1. *PLoSOne* 2017 (in press)
2. Micol, JB, Pastore, A, Inoue, D, Duployez, N, Kim, E, Lee, SCW, Durham, BH, Chung, YR, Cho, H, Zhang, XJ, Yoshimi, A, Krivtsov, A, Koche, R, Solary, E, Sinha, A, Preudhomme, C, **Abdel-Wahab, O**. ASXL2 is essential for normal hematopoiesis and a haploinsufficient tumor suppressor in leukemia. *Nature Communications* 2017 (In press)
2. Xia S, Lin R, Jin L, Zhao L, Kang HB, Pan Y, Liu S, Qian G, Qian Z, Konstantakou E, Zhang B, Dong JT, Chung YR, **Abdel-Wahab O**, Merghoub T, Zhou L, Kudchadkar RR, Lawson DH, Khoury HJ, Khuri FR, Boise LH, Lonial S, Lee BH, Pollack BP, Arbiser JL, Fan J, Lei QY, Chen J. Prevention of Dietary-Fat-Fueled Ketogenesis Attenuates BRAF V600E Tumor Growth. Cell Metab. 2017 Jan 9. pii: S1550-4131(16)30643-X. doi: 10.1016/j.cmet.2016.12.010. [Epub ahead of print] PubMed PMID: 28089569.
3. Momtaz P, Pentsova E, **Abdel-Wahab O**, Diamond E, Hyman D, Merghoub T, You D, Gasmi B, Viale A, Chapman PB. Quantification of tumor-derived cell free DNA(cfDNA) by digital PCR (DigPCR) in cerebrospinal fluid of patients with BRAFV600 mutated malignancies. Oncotarget. 2016 Dec 20;7(51):85430-85436. doi: 10.18632/oncotarget.13397. PubMed PMID: 27863426.
4. Guryanova, O, Shank, K, Spitzer, B, Luciani, L, Koche, R, Garrett-Bakelman, F, Ganzel, C, Durham, B, Mohanty, A, Hoermann, G, Rivera, S, Chramiec, A, Pronier, E, Bastian, L, Keller, M, Tovbin, D, Loizou, E, Weinstein, A, Rodriguez Gonzalez, A, Lieu, Y, Rowe, J, Pastore, F, McKenney, A, Krivtsov, A, Sperr, WR, Cross, J, Mason, C, Tallman, M, Arcila, ME, **Abdel-Wahab, O,** Armstrong, S, Kubicek, S, Staber, P, Gönen, M, Paietta, E, Melnick, A, Nimer, S, Mukherjee, S, Levine, RL. DNMT3A R882 mutations promote anthracycline resistance in acute myeloid leukemia through impaired nucleosome remodeling. Nat Med. 2016 Dec;22(12):1488-1495. doi: 10.1038/nm.4210. Epub 2016 Nov 14. PubMed PMID: 27841873; PubMed Central PMCID: PMC5359771.
5. Berman E, Jhanwar S, Hedvat C, Arcila ME, **Wahab OA**, Levine R, Maloy M, Ma W, Albitar M. Resistance to imatinib in patients with chronic myelogenous leukemia and the splice variant BCR-ABL1(35INS). Leuk Res. 2016 Oct;49:108-12. doi: 10.1016/j.leukres.2016.08.006. Epub 2016 Aug 12. PubMed PMID: 27658269.
6. Diamond EL, **Abdel-Wahab O**, Durham BH, Dogan A, Ozkaya N, Brody L, Arcila M, Bowers C, Fluchel M. Anakinra as efficacious therapy for two cases of intracranial Erdheim-Chester disease. Blood. 2016 Aug 17. pii: blood-2016-06-725143. [Epub ahead of print] PubMed PMID: 27535996.
7. Béguelin W, Teater M, Gearhart MD, Calvo Fernández MT, Goldstein RL, Cárdenas MG, Hatzi K, Rosen M, Shen H, Corcoran CM, Hamline MY, Gascoyne RD, Levine RL, **Abdel-Wahab O**, Licht JD, Shaknovich R, Elemento O, Bardwell VJ, Melnick AM. EZH2 and BCL6 Cooperate to Assemble CBX8-BCOR Complex to Repress Bivalent Promoters, Mediate Germinal Center Formation and Lymphomagenesis. Cancer Cell. 2016 Aug 8;30(2):197-213. doi: 10.1016/j.ccell.2016.07.006. PubMed PMID: 27505670.
8. Diamond EL, Hatzoglou V, Patel S, **Abdel-Wahab O**, Rampal R, Hyman DM, Holodny AI, Raj A. Diffuse reduction of cerebral grey matter volumes in Erdheim-Chester disease. Orphanet J Rare Dis. 2016 Aug 2;11(1):109. doi: 10.1186/s13023-016-0490-3. PubMed PMID: 27484739; PubMed Central PMCID: PMC4971748.
9. Shi H, Yamamoto S, Sheng M, Bai J, Zhang P, Chen R, Chen S, Shi L, **Abdel-Wahab O**, Xu M, Zhou Y, Yang FC. ASXL1 plays an important role in erythropoiesis. Sci Rep. 2016 Jun 29;6:28789. doi: 10.1038/srep28789. PubMed PMID: 27352931; PubMed Central PMCID: PMC4926121.
10. Getta BM, Woo KM, Devlin S, Park JH, **Abdel-Wahab O**, Saven A, Rai K, Tallman MS. Treatment outcomes and secondary cancer incidence in young patients with hairy cell leukaemia. Br J Haematol. 2016 Jun 28. doi: 10.1111/bjh.14207. [Epub ahead of print] PubMed PMID: 27351754.

11. Lu R, Wang P, Parton T, Zhou Y, Chrysovergis K, Rockowitz S, Chen WY, **Abdel-Wahab O**, Wade PA, Zheng D, Wang GG. Epigenetic Perturbations by Arg882-Mutated DNMT3A Potentiate Aberrant Stem Cell Gene-Expression Program and Acute Leukemia Development. Cancer Cell. 2016 Jul 11;30(1):92-107. doi: 10.1016/j.ccell.2016.05.008. Epub 2016 Jun 23. PubMed PMID: 27344947; PubMed Central PMCID: PMC4945461.
12. Zhang, P, Xing, C, Rhodes, SD, He, Y, Deng, K, Li, Z, He, F, Zhu, C, Nguyen, L, Zhou, Y, Chen, S, Mohammad, KS, Guise, TA, **Abdel-Wahab, O**, Xu, M, Wang, QF, Yang, FC. Loss of Asxl1 Alters Self-Renewal and Cell Fate of Bone Marrow Stromal Cell, Leading to Bohring-Opitz-like Syndrome in Mice. Stem Cell Reports. 2016 May 24. pii: S2213-6711(16)30038-8. doi: 10.1016/j.stemcr.2016.04.013. [Epub ahead of print] PubMed PMID: 27237378
13. Lee SC, Dvinge H, Kim E, Cho H, Micol JB, Chung YR, Durham BH, Yoshimi A, Kim YJ, Thomas M, Lobry C, Chen CW, Pastore A, Taylor J, Wang X, Krivtsov A, Armstrong SA, Palacino J, Buonamici S, Smith PG, Bradley RK, **Abdel-Wahab O**. Modulation of splicing catalysis for therapeutic targeting of leukemia with mutations in genes encoding spliceosomal proteins. Nat Med. 2016 Jun;22(6):672-8. doi: 10.1038/nm.4097. Epub 2016 May 2. PubMed PMID: 27135740; PubMed Central PMCID: PMC4899191.
14. He J (equal contributor), **Abdel-Wahab O (equal contributor)**, Nahas MK (equal contributor), Wang K, Rampal RK, Intlekofer AM, Patel J, Krivstov A, Frampton GM, Young LE, Zhong S, Bailey M, White JR, Roels S, Deffenbaugh J, Fichtenholtz A, Brennan T, Rosenzweig M, Pelak K, Knapp KM, Brennan KW, Donahue AL, Young G, Garcia L, Beckstrom ST, Zhao M, White E, Banning V, Buell J, Iwanik K, Ross JS, Morosini D, Younes A, Hanash AM, Paietta E, Roberts K, Mullighan C, Dogan A, Armstrong SA, Mughal T, Vergilio JA, Labrecque E, Erlich R, Vietz C, Yelensky R, Stephens PJ, Miller VA, van den Brink MR, Otto GA, Lipson D, Levine RL. Integrated genomic DNA/RNA profiling of hematologic malignancies in the clinical setting. *Blood*. 2016 Jun 16;127(24):3004-14. doi: 10.1182/blood-2015-08-664649. Epub 2016 Mar 10. PubMed PMID: 26966091.
15. Duployez N, Marceau-Renaut A, Boissel N, Petit A, Bucci M, Geffroy S, Lapillonne H, Renneville A, Ragu C, Figeac M, Celli-Lebras K, Lacombe C, Micol JB, **Abdel-Wahab O**, Cornillet P, Ifrah N, Dombret H, Leverger G, Jourdan E, Preudhomme C. Comprehensive mutational profiling of core binding factor acute myeloid leukemia. Blood. 2016 May 19;127(20):2451-9. doi: 10.1182/blood-2015-12-688705. Epub 2016 Mar 15. PubMed PMID: 26980726.
16. Taggart J, Ho TC, Amin E, Xu H, Barlowe TS, Perez AR, Durham BH, Tivnan P, Okabe R, Chow A, Vu L, Park SM, Prieto C, Famulare C, Patel M, Lengner CJ, Verma A, Roboz G, Guzman M, Klimek VM, **Abdel-Wahab O**, Leslie C, Nimer SD, Kharas MG. MSI2 is required for maintaining activated myelodysplastic syndrome stem cells. Nat Commun. 2016 Feb 22;7:10739. doi: 10.1038/ncomms10739. PubMed PMID: 26898884; PubMed Central PMCID: PMC4764878.
17. Luskin, MR, Lee, J-W, Fernandez, HF, **Abdel-Wahab, O**, Bennett, JM, Ketterling, RP, Lazarus, HM, Levine, RL, Litzow, MR, Paietta, EM, Patel, JP, Racevskis, J, Rowe, JM, Tallman, MS, Sun, Z, Luger, SM. Benefit of high dose daunorubicin in AML induction extends across cytogenetic and molecular groups: updated analysis of E1900. *Blood*. 2016 Mar 24;127(12):1551-8. doi: 10.1182/blood-2015-07-657403. PubMed PMID: 26755712; PubMed Central PMCID: PMC4807422.
18. Antony-Debré, I, Duployez, N, Bucci, M, Geffroy, S, Micol, J-B, Renneville, A, Boissel, N, Dhédin, N, Réa, D, Nelken, B, Berthon, C, Leblanc, T, Mozziconacci, M-J, Favier, R, Heller, PG, **Abdel-Wahab, O**, Raslova, H, Latger-Cannard, V and Preudhomme, C. Somatic mutations associated with leukemic progression of FPD/AML. *Leukemia*. 2016 Apr;30(4):999-1002. doi: 10.1038/leu.2015.236. PubMed PMID: 26316320.
19. Guryanova, O, Lieu, Y, Garrett-Bakelman, FE, Spitzer, B, Glass, J, Shank, K, Valencia Martinez, AB, Rivera, S, Durham, B, Rapaport, F, Keller, M, Pandey, S, Bastian, L, Tovbin, D, Weinstein, A, Teruya-Feldstein, J, **Abdel-Wahab, O**, Santini, V, Mason, C, Melnick, A, Mukherjee, S, Levine, RL. Dnmt3a Regulates Myeloproliferation and Liver-Specific Expansion of Hematopoietic Stem and Progenitor Cells. *Leukemia*. 2016 May;30(5):1133-42. doi: 10.1038/leu.2015.358. PubMed PMID: 26710888; PubMed Central PMCID: PMC4856586.

20. Zhang L, Tran NT, Su H, Wang R, Lu Y, Tang H, Aoyagi S, Guo A, Khodadadi-Jamayran A, Zhou D, Qian K, Hricik T, Côté J, Han X, Zhou W, Laha S, **Abdel-Wahab O**, Levine RL, Raffel G, Liu Y, Chen D, Li H, Townes T, Wang H, Deng H, Zheng YG, Leslie C, Luo M, Zhao X. Cross-talk between PRMT1-mediated methylation and ubiquitylation on RBM15 controls RNA splicing. *Elife*. 2015 Nov 17;4. pii: e07938. doi: 10.7554/eLife.07938. [Epub ahead of print] PubMed PMID: 26575292.
21. Wen JQ, Yang Q, Goldenson B, Malinge S, Lasho T, Schneider RK, Breyfogle LJ, Schultz R, Gilles L, Koppikar P, **Abdel-Wahab O**, Pardanani A, Stein B, Gurbuxani S, Mullally A, Levine RL, Tefferi A, Crispino JD. Targeting megakaryocytic-induced fibrosis in myeloproliferative neoplasms by AURKA inhibition. *Nat Med.* 2015 Nov 16. doi: 10.1038/nm.3995. [Epub ahead of print] PubMed PMID: 26569382.
22. Diamond EL, Durham BH, Haroche J, Yao Z, Ma J, Parikh SA, Wang Z, Choi J, Kim E, Cohen-Aubart F, Lee SC, Gao Y, Micol JB, Campbell P, Walsh MP, Sylvester B, Dolgalev I, Aminova O, Heguy A, Zappile P, Nakitandwe J, Ganzel C, Dalton JD, Ellison DW, Estrada-Veras J, Lacouture M, Gahl WA, Stephens PJ, Miller VA, Ross JS, Ali SM, Briggs SR, Fasan O, Block J, Heritier S, Donadieu J, Solit DB, Hyman DM, Baselga J, Janku F, Taylor BS, Park CY, Amoura Z, Dogan A, Emile JF, Rosen N, Gruber TA, **Abdel-Wahab O**. Diverse and Targetable Kinase Alterations Drive Histiocytic Neoplasms. Cancer Discov. 2015 Nov 13. pii: CD-15-0913. [Epub ahead of print] PubMed PMID: 26566875.
23. LaFave LM, Béguelin W, Koche R, Teater M, Spitzer B, Chramiec A, Papalexi E, Keller MD, Hricik T, Konstantinoff K, Micol JB, Durham B, Knutson SK, Campbell JE, Blum G, Shi X, Doud EH, Krivtsov AV, Chung YR, Khodos I, de Stanchina E, Ouerfelli O, Adusumilli PS, Thomas PM, Kelleher NL, Luo M, Keilhack H, **Abdel-Wahab O**, Melnick A, Armstrong SA, Levine RL. Loss of BAP1 function leads to EZH2-dependent transformation. Nat Med. 2015 Nov;21(11):1344-9. doi: 10.1038/nm.3947. Epub 2015 Oct 5. PubMed PMID: 26437366; PubMed Central PMCID: PMC4636469.
24. Tiacci E, Park JH, De Carolis L, Chung SS, Broccoli A, Scott S, Zaja F, Devlin S, Pulsoni A, Chung YR, Cimminiello M, Kim E, Rossi D, Stone RM, Motta G, Saven A, Varettoni M, Altman JK, Anastasia A, Grever MR, Ambrosetti A, Rai KR, Fraticelli V, Lacouture ME, Carella AM, Levine RL, Leoni P, Rambaldi A, Falzetti F, Ascani S, Capponi M, Martelli MP, Park CY, Pileri SA, Rosen N, Foà R, Berger MF, Zinzani PL, **Abdel-Wahab O (equal contributor)**, Falini B (equal contributor; corresponding), Tallman MS (equal contributor). Targeting Mutant BRAF in Relapsed or Refractory Hairy-Cell Leukemia. N Engl J Med. 2015 Oct 29;373(18):1733-47. doi: 10.1056/NEJMoa1506583. Epub 2015 Sep 9. PubMed PMID: 26352686.
25. Fong CY, Gilan O, Lam EY, Rubin AF, Ftouni S, Tyler D, Stanley K, Sinha D, Yeh P, Morison J, Giotopoulos G, Lugo D, Jeffrey P, Lee SC, Carpenter C, Gregory R, Ramsay RG, Lane SW, **Abdel-Wahab O**, Kouzarides T, Johnstone RW, Dawson SJ, Huntly BJ, Prinjha RK, Papenfuss AT, Dawson MA. BET inhibitor resistance emerges from leukaemia stem cells. Nature. 2015 Sep 24;525(7570):538-42. doi: 10.1038/nature14888. Epub 2015 Sep 14. PubMed PMID: 26367796.
26. Yao, Z, Torres, NM, Tao, A, Gao, Y, Luo, L, Li, Q, De Stanchina, E, **Abdel-Wahab, O**, Solit, D, Poulikakos, P, and Rosen, N. BRAF mutants evade ERK dependent feedback by different mechanisms that determine their sensitivity to pharmacologic inhibition. Cancer Cell. 2015 Sep 14;28(3):370-83. doi: 10.1016/j.ccell.2015.08.001. PubMed PMID: 26343582; PubMed Central PMCID: PMC4894664.
27. Kang, H-B, Fan, J, Shan, C, Elf, S, Ji, Q, Seo, JH, Arbiser, J, Miziorko, HM, **Abdel-Wahab, O**, Fröhling, S, Scholl, C, Tamayo, P, Barbie, DA, Lonial, S, Khoury, HJ, Khuri, FR, Lee, BH, He, C, Kang, S, and Chen, J. 3-hydroxy-3-methylglutaryl-CoA lyase is a synthetic lethal partner of BRAF V600E in human melanoma. *Mol Cell* 2015 Jul 1. pii: S1097-2765(15)00438-4. doi: 10.1016/j.molcel.2015.05.037. [Epub ahead of print] PubMed PMID: 26145173.
28. Watts, JM, Kishtagari, A, Hsu, M, Stein, EM, Park, JH, Lacouture, ME, Postow, M, Feldstein, J, **Abdel-Wahab, O,** Devlin, S, and Tallman, MS. Melanoma and non-melanoma skin cancers in hairy cell leukemia: a SEER population analysis and the 30-year experience at Memorial Sloan Kettering Cancer Center. *Br J Haematol*. 2015 Oct;171(1):84-90. doi: 10.1111/bjh.13528. PubMed PMID: 26115047; PubMed Central PMCID: PMC4766022.
29. Walter, RB, Othus, M, Paietta, EM, Racevskis, J, Fernandez, HF, Lee, J-

W, Sun, Z, Tallman, MS, Patel, J, Gönen, M, **Abdel-Wahab, O**, Levine, RL, and Estey, EH. Effect of Genetic Profiling on Prediction of Therapeutic Resistance and Survival in Adult Acute Myeloid Leukemia. *Leukemia*. 2015 Oct;29(10):2104-7. doi: 10.1038/leu.2015.76. PubMed PMID: 25772026; PubMed Central PMCID: PMC4573365.

30. Dietrich S, Hüllein J, Lee SC, Hutter B, Gonzalez D, Jayne S, Dyer MJ, Oleś M, Else M, Liu X, Słabicki M, Wu B, Troussard X, Dürig J, Andrulis M, Dearden C, von Kalle C, Granzow M, Jauch A, Fröhling S, Huber W, Meggendorfer M, Haferlach T, Ho AD, Richter D, Brors B, Glimm H, Matutes E, **Abdel Wahab O**, Zenz T. Recurrent CDKN1B (p27) mutations in hairy cell leukemia. Blood. 2015 Jun 11. pii: blood-2015-04-643361. [Epub ahead of print] PubMed PMID: 26065650.
31. Kim, E, Ilagan, JO (co-first author), Liang, Y (co-first author), Daubner, GM (co-first author), Lee, S, Ramakrishnan, A, Li, Y, Chung, YR, Micol, J-B, Murphy, M, Cho, H, Kim, M-K, Zebari, AS, Buonamici, S, Smith, P, Deeg, HJ, Lobry, C, Aifantis, I, Modis, Y, Allain, F.H.-T., Halene, S (co-corresponding), Bradley, RK (co-corresponding), **Abdel-Wahab, O** (co-corresponding)**.** *SRSF2* Mutations Contribute to Myelodysplasia Through Mutant-Specific Effects on Exon Recognition. *Cancer Cell* . 2015 May 11;27(5):617-30. doi: 10.1016/j.ccell.2015.04.006. PubMed PMID: 25965569; PubMed Central PMCID: PMC4429920.
32. Duployez N, Micol JB, Boissel N, Petit A, Geffroy S, Bucci M, Lapillonne H, Renneville A, Leverger G, Ifrah N, Dombret H, **Abdel-Wahab O**, Jourdan E, Preudhomme C. Unlike ASXL1 and ASXL2 mutations, ASXL3 mutations are rare events in acute myeloid leukemia with t(8;21). *Leuk Lymphoma*. 2015 Apr 9:1-6. [Epub ahead of print] PubMed PMID: 25856206.
33. Meldi, K, Qin, T, Buchi, F, Droin, N, Sotzen, J, Micol, J-B, Selimoglu-Buet, D, Masala, E, Allione, B, Gioia, D, Poloni, A, Lunghi, M, Solary, E, **Abdel-Wahab, O**, Santini, V, and Figueroa, ME. Specific molecular signatures predict decitabine response in chronic myelomonocytic leukemia. *J Clin Invest*. 2015 May;125(5):1857-72. doi: 10.1172/JCI78752. PubMed PMID: 25822018; PubMed Central PMCID: PMC4611703.
34. Kleppe, M, Kwak, M, Koppikar, P, Riester, M, Keller, M, Bastian, L, Hricik, T, Bhagwat, N, **Abdel-Wahab, O**, Rampal, R, Marubayashi, S, Chen, JJ, Romanet, V, Fridman, J, Bromberg, J, Teruya-Feldstein, J, Murakami, M, Radimerski, T, Michor, F, Fan, F, and Levine, RL. JAK-STAT Pathway Activation in Malignant and Non-Malignant Cells Contributes to MPN Pathogenesis and Therapeutic Response. *Cancer Discovery* 2015 Mar;5(3):316-31. doi: 10.1158/2159-8290.CD-14-0736. PubMed PMID: 25572172; PubMed Central PMCID: PMC4355105.
35. Rampal, R, Alkalin, A, Madzo, J, Vasanthakumar, A, Pronier, E, Patel, J, Li, Y, Ahn, J, **Abdel-Wahab, O,** Shih, A, Lu, C, Ward, PS, Tsai, JJ, Hricik, T, Tosello, V, Tallman, JE, Zhao, X, Daniels, D, Dai, Q, Cimminio, L, Aifantis, I, He, C, Fuks, F, Tallman, MS, Ferrando, A, Nimer, S, Paietta, E, Thompson, CB, Licht, JD, Mason, C, Godley, LA, Melnick, A, Figueroa, ME, and Levine, RL. DNA hydroxymethylation profiling reveals that WT1 mutations result in loss of TET2 function in acute myeloid leukemia. *Cell Reports* 2014 Dec 11;9(5):1841-55. doi: 10.1016/j.celrep.2014.11.004. PubMed PMID: 25482556; PubMed Central PMCID: PMC4267494.
36. Inoue, D, Kitaura, J, Matsui, H, Hou, H-A, Chou, W-C, Nagamachi, A, Kawabata, K, Togami, K, Nagase, R, Horikawa, S, Saika, M, Micol, J-B, Hayashi, Y, Harada, Y, Harada, H, Inaba, T, Tien, H-F, **Abdel-Wahab, O**, and Kitamura, T. *SETBP1* Mutations Drive Leukemic Transformation in *ASXL1*-Mutated MDS. *Leukemia* 2015 Apr;29(4):847-57. doi: 10.1038/leu.2014.301. PubMed PMID: 25306901; PubMed Central PMCID: PMC4501574.
37. Hyman, DM, Diamond, EL, Vibat, CRT, Hassaine, L, Poole, JC, Patel, M, Holley, VR, Cabrilo, G, Lu, TT, Arcila, ME, Chung, YR, Rampal, R, Lacouture, ME, Rosen, N, Meric-Bernstam, F, Baselga, J, Kurzrock, R, Erlander, MG, Janku, F, **Abdel-Wahab, O**. Prospective Blinded Study of BRAFV600E Mutation Detection in Cell-Free DNA of Patients with Systemic Histiocytic Disorders. *Cancer Discovery* 2015 Jan;5(1):64-71. doi: 10.1158/2159-8290.CD-14-0742. PubMed PMID: 25324352.
38. Rampal, R, Ahn, J, **Abdel-Wahab, O**, Lipson, D, Otto, G, Hricik, T, McKenney, A, Wang, K, Nahas, M, Chung, YR, Pandey, S, van den Brink, M, Armstrong, S, Dogan, A, Intelkofer, A,

Mansouri, T, Park, C, Vertovsek, S, Stephens, P, Miller, V, and Levine, RL. Genomic and Functional Analysis of Leukemic Transformation of Myeloproliferative Neoplasms. *PNAS* 2014 Dec 16;111(50):E5401-10. doi: 10.1073/pnas.1407792111. PubMed PMID: 25516983; PubMed Central PMCID: PMC4273376.

39. Emile, J-F, Diamond, E, Hélias-Rodzewicz, Z, Cohen Aubart, F, Charlotte, F, Hyman, DM, Kim, E, Rampal, R, Patel, M, Ganzel, C, Aumann, S, Faucher, G, Le Gall, C, Leroy, K, Colombat, M, Kahn, JE, Trad, S, Nizard, P, Donadieu, J, Taly, V, Amoura, Z, **Abdel-Wahab, O (Equal contributor; co-corresponding)**, and Haroche, J. Recurrent RAS and PIK3CA mutations in Erdheim-Chester Disease. *Blood*. 2014 August. PubMed PMID: 25150293.
40. Micol, JB, Duployez, N, Boissel, N, Petit, A, Sandrine, G, Nibourel, O, Lacombe, C, Lapillonne, H, Etancelin, P, Figeac, M, Renneville, A, Castaigne, S, Leverger, G, Ifrah, NH, Dombret, H, Preudhomme, C, **Abdel-Wahab, O (co-corresponding)**, and Jourdan, E. Frequent *ASXL2* mutations in acute myeloid leukemia patients with t(8;21)/RUNX1-RUNX1T1 chromosomal translocations. *Blood*. 2014 August. PubMed PMID: 24973361.
41. Guzman ML, Yang N, Sharma KK, Balys M, Corbett CA, Jordan CT, Becker MW, Steidl U, **Abdel-Wahab O**, Levine RL, Marcucci G, Roboz GJ, Hassane DC. Selective activity of the histone deacetylase inhibitor AR-42 against leukemia stem cells: a novel potential strategy in acute myeloid leukemia. *Mol Cancer Ther*. 2014 Jun16. PubMed PMID: 24934933
42. Rampal, R (**co-first author**), Al-Shahrour, F (**co-first author**), **Abdel-Wahab, O** (**co-first author**), Patel, J, Brunel, J-P, Mermel, CH, Bass, AJ, Pretz, J, Ahn, J, Hricik, T, Kilpivaara, O, Wadleigh, M, Busque, L, Gilliland, DG, Golub, TR, Ebert, BL, and Levine, RL. Integrated genomic analysis illustrates the central role of JAK-STAT pathway activation in myeloproliferative neoplasm pathogenesis. *Blood.* 2014 April 16. PubMed PMID: 24740812 PMCID: PMC4041169.
43. Chung, S, Kim, E, Park, JH, Chung, YR, Lito, P, Feldstein, J, Hu, W, Beguilin, W, Monette, S, Duy, C, Rampal, R, Telis, L, Patel, M, Kim, MK, Melnick, AM, Rosen, N, Tallman, MS, Park, CY, and **Abdel-Wahab, O**. Hematopoietic Stem Cell Origin of *BRAF*V600E Mutations in Hairy Cell Leukemia. *Science Translational Medicine.* 2014 May 28. PMID: 24871132.
44. **Abdel-Wahab O**, Klimek VM, Gaskell A, Viale A, Cheng D, Kim E, Rampal R, Bluth M, Harding JJ, Callahan MK, Merghoob T, Berger MF, Solit DB, Rosen N, Levine RL, Chapman PB. Efficacy of intermittent combined RAF and MEK inhibition in a patient with concurrent BRAF and NRAS mutant malignancies. *Cancer Discov.* 2014 Mar 3. PMID: 24589925.
45. Akbay EA, Moslehi J, Christensen CL, Saha S, Tchaicha JH, Ramkissoon SH, Stewart KM, Carretero J, Kikuchi E, Zhang H, Cohoon TJ, Murray S, Liu W, Uno K, Fisch S, Jones K, Gurumurthy S, Gliser C, Choe S, Keenan M, Son J, Stanley I, Losman JA, Padera R, Bronson RT, Asara JM, **Abdel-Wahab O**, Amrein PC, Fathi AT, Danial NN, Kimmelman AC, Kung AL, Ligon KL, Yen KE, Kaelin WG Jr, Bardeesy N, Wong KK. D-2-hydroxyglutarate produced by mutant IDH2 causes cardiomyopathy and neurodegeneration in mice. 2014 Mar 1. PMID: 24589777. PMCID: PMC3950345.
46. Inoue, D, Kitaura, J, Togami, K, Nishimura, K, Enomoto, Y, Uchida, T, Kagiyama, Y, Kawabata, KC, Nakahara, F, Izawa, K, Oki, T, Maehara, A, Isobe, M, Tsuchiya, A, Harada, Y, Harada, H, Ochiya, T, Aburatani, H, Kimura, H, Thol, F, Heuser, M, Levine, RL, Abdel-Wahab, O, and Kitamura, T. Myelodysplastic syndromes are induced by histone methylation–altering *ASXL1* mutations. *J Clin Invest.* 2013 Nov 1. PMID: 24216483. PMCID: PMC3809801.
47. **Abdel-Wahab, O**, Gao, J, Adli, MM, Dey, A, Trimarchi, T, Chung, YR, Kuscu, C, Hricik, T, Ndiaye-Lobry, D, La Fave, LM, Koche, R, Shih, AH, Guryanova, OA, Kim, E, Pandey, S, Shin, JY, Liu, J, Bhatt, PK, Monette, S, Zhao, X, Park, CY, Bernstein, BE, Aifantis, I, Levine, RL. Deletion of Asxl1 Results in Myelodysplasia and Severe Developmental Defects in Vivo. *J Exp Med* 2013 Nov 18;210(12):2641-59.doi: 10.1084/jem.20131141. Epub 2013 Nov 11. PubMed PMID: 24218140.
48. Diamond EL, **Abdel-Wahab O**, Pentsova E, Borsu L, Chiu A, Teruya-Feldstein J, Hyman DM, Rosenblum M. Detection of an NRAS mutation in Erdheim-Chester disease. Blood. 2013 Aug 8;122(6):1089-91. doi: 10.1182/blood-2013-02-482984. PubMed PMID: 23929840.
49. Oaks JJ, Santhanam R, Walker CJ, Roof S, Harb JG, Ferenchak G, Eisfeld AK, Van Brocklyn JR, Briesewitz R, Saddoughi SA, Nagata K, Bittman R, Caligiuri MA, **Abdel-Wahab O**, Levine R, Arlinghaus RB, Quintas-Cardama A, Goldman JM, Apperley J, Reid A, Milojkovic D, Ziolo

MT, Marcucci G, Ogretmen B, Neviani P, Perrotti D. Antagonistic activities of the immunomodulator and PP2A-activating drug FTY720 (Fingolimod, Gilenya) in Jak2-driven hematologic malignancies. Blood. 2013 Aug 7. [Epub ahead of print] PubMed PMID: 23926298.
50. Béguelin W, Popovic R, Teater M, Jiang Y, Bunting KL, Rosen M, Shen H, Yang SN, Wang L, Ezponda T, Martinez-Garcia E, Zhang H, Zheng Y, Verma SK, McCabe MT, Ott HM, Van Aller GS, Kruger RG, Liu Y, McHugh CF, Scott DW, Chung YR, Kelleher N, Shaknovich R, Creasy CL, Gascoyne RD, Wong KK, Cerchietti L, Levine RL, **Abdel-Wahab O**, Licht JD, Elemento O, Melnick AM. EZH2 is required for germinal center formation and somatic EZH2 mutations promote lymphoid transformation. Cancer Cell. 2013 May 13;23(5):677-92. doi: 10.1016/j.ccr.2013.04.011. PubMed PMID: 23680150; PubMed Central PMCID: PMC3681809.
51. DiNardo CD, Propert KJ, Loren AW, Paietta E, Sun Z, Levine RL, Straley KS, Yen K, Patel JP, Agresta S, **Abdel-Wahab O**, Perl AE, Litzow MR, Rowe JM, Lazarus HM, Fernandez HF, Margolis DJ, Tallman MS, Luger SM, Carroll M. Serum 2-hydroxyglutarate levels predict isocitrate dehydrogenase mutations and clinical outcome in acute myeloid leukemia. Blood. 2013 Jun 13;121(24):4917-24. doi: 10.1182/blood-2013-03-493197. Epub 2013 May 2. PubMed PMID: 23641016; PubMed Central PMCID: PMC3682342.
52. Padron E, Painter JS, Kunigal S, Mailloux AW, McGraw K, McDaniel JM, Kim E, Bebbington C, Baer M, Yarranton G, Lancet J, Komrokji RS, **Abdel-Wahab O**, List AF, Epling-Burnette PK. GM-CSF-dependent pSTAT5 sensitivity is a feature with therapeutic potential in chronic myelomonocytic leukemia. Blood. 2013 Jun 20;121(25):5068-77. doi: 10.1182/blood-2012-10-460170. Epub 2013 Apr 30. PubMed PMID: 23632888.
53. Quintás-Cardama A* (co-first authors), **Abdel-Wahab O* (Co-first authors)**, Manshouri T, Kilpivaara O, Cortes J, Roupie AL, Zhang SJ, Harris D, Estrov Z, Kantarjian H, Levine RL, Verstovsek S. Molecular analysis of patients with polycythemia vera or essentialthrombocythemia receiving pegylated interferon alpha-2a. Blood. 2013 Jun 19. [Epub ahead of print] PubMed PMID: 23782935.
54. Wei Y, Dimicoli S, Bueso-Ramos C, Chen R, Yang H, Neuberg D, Pierce S, Jia Y, Zheng H, Wang H, Wang X, Nguyen M, Wang SA, Ebert B, Bejar R, Levine R, **Abdel-Wahab O**, Kleppe M, Ganan-Gomez I, Kantarjian H, Garcia-Manero G. Toll-like receptor alterations in myelodysplastic syndrome. Leukemia. 2013 Jun 14. doi: 10.1038/leu.2013.180. [Epub ahead of print] PubMed PMID: 23765228.
55. Reavie, L, Buckley, SM, Loizou, E, Takeishi, S, Aranda-Orgilles, B, Ndiaye-Lobry, D, **Abdel-Wahab, O,** Ibrahim, S, Nakayama, KI, Aifantis, I. Regulation of c-Myc Ubiquitination Controls Chronic Myelogenous Leukemia Initiation and Progression. *Cancer Cell,* 2013 Mar 18;23(3)362-375.
56. Ramos P, Casu C, Gardenghi S, Breda L, Crielaard BJ, Guy E, Marongiu MF, Gupta R, Levine RL, **Abdel-Wahab O**, Ebert BL, Van Roolien N, Ghaffari S, Grady RW, Giardina PJ, Rivella S. Macrophages support pathological erythropoiesis in polycythemia vera and β-thalassemia. *Nat Med.* 2013 Mar 17. doi: 10.1038/nm.3126. [Epub ahead of print]. PMID: 23502961
57. Lobry C, Ntziachristos P, Ndiaye-Lobry D, Oh P, Cimmino L, Zhu N, Araldi E, Hu W, Freund J, **Abdel-Wahab O**, Ibrahim S, Skokos D, Armstrong SA, Levine R.L., Park CY, Aifantis I. Notch pathway activation targets AML-initiating cell homeostasis and differentiation. *J Exp Med.* 2013 Feb 11;210(2):301-19. PMCID: PMC3570103
58. Shih AH, Chung SS, Dolezal EK, Zhang SJ, **Abdel-Wahab OI**, Park CY, Nimer SD, Levine RL, Klimek VM. Mutational analysis of therapy-related myelodysplastic syndromes and acute myelogenous leukemia. *Haematologica*. 2013 Jan 24 [Epub ahead of print] PMID: 23349305.
59. Gautier EL, Westerterp M, Bhagwat N, Cremers S, Shih A, **Abdel-Wahab O**, Lütjohann D, Randolph GJ, Levine RL, Tall AR, Yvan-Charvet L. HDL and Glut1 inhibition reverse a hypermetabolic state in mouse models of myeloproliferative disorders. *J Exp Med*. 2013 Feb 11;210(2):339-53. PMCID: PMC3570097.
60. Ward PS, Lu C, Cross JR, **Abdel-Wahab O**, Levine RL, Schwartz GK, Thompson CB. The Potential for Isocitrate Dehydrogenase Mutations to Produce 2-Hydroxyglutarate Depends on Allele Specificity and Subcellular Compartmentalization. *J Biol Chem*. 2013 Feb 8;288(6):3804-15. PMCID: PMC3567635
61. Pollyea DA, Zehnder J, Coutre S, Gotlib J, Gallegos L, **Abdel-Wahab O**, Greenberg P, Zhang B, Liedtke M, Berube C, Levine R, Mitchell BS, Medeiros BC. Sequential azacitidine plus

lenalidomide combination for elderly patients with untreated acute myeloid leukemia. *Haematologica*. 2012 Dec 14 [Epub ahead of print] PMID: 23242596.
62. Baljevic M, **Abdel-Wahab O**, Rampal R, Maslak PG, Klimek VM, Rosenblat TL, Douer D, Levine RL, Tallman MS. Translocation t(11;17) in de novo Myelodysplastic Syndrome Not Associated with Acute Myeloid or Acute Promyelocytic Leukemia. *Acta Haematologica*. 2013;129(1):48-54. PMID: 23147462.
63. Callahan MK, Rampal R, Harding JJ, Klimek VM, Chung YR, Merghoub T, Wolchok, JD, Solit DB, Rosen N, **Abdel-Wahab O**, Levine RL, Chapman PB. Progression of RAS-Mutant Leukemia during RAF Inhibitor Treatment. *N Engl J Med.* 2012 Dec 13;367(24):2316-21. PMID: 23134356.
64. Hakimi AA, Chen YB, Wren J, Gonen M, **Abdel-Wahab O**, Heguy A, Liu H, Takeda S, Tickoo SK, Reuter VE, Voss MH, Motzer RJ, Coleman JA, Cheng EH, Russo P, Hsieh JJ. Clinical and Pathologic Impact of Select Chromatin-modulating Tumor Suppressors in Clear Cell Renal Cell Carcinoma. *Eur Urol.* 2012 Sep 27. pii: S0302-2838(12)01028-7. doi: 10.1016/j.eururo.2012.09.005. [Epub ahead of print]. PMID: 23036577.
65. Diab A, Zickl L, **Abdel-Wahab O**, Jhanwar S, Gulam MA, Panageas KS, Patel JP, Jurcic J, Maslak P, Paietta E, Mangan JK, Carroll M, Fernandez HF, Teruya-Feldstein J, Luger SM, Douer D, Litzow MR, Lazarus HM, Rowe JM, Levine RL, Tallman MS. Acute myeloid leukemia with translocation t(8;16) presents with features which mimic acute promyelocytic leukemia and is associated with poor prognosis. *Leuk Res*. 2013 Jan;37(1):32-6. PMID: 23102703.
66. Busque L, Patel JP, Figueroa ME, Vasanthakumar A, Provost S, Hamilou Z, Mollica L, Li J, Viale A, Heguy A, Hassimi M, Socci N, Bhatt PK, Gonen M, Mason CE, Melnick A, Godley LA, Brennan CW, **Abdel-Wahab O* (co-last author),** Levine RL. Recurrent somatic TET2 mutations in normal elderly individuals with clonal hematopoiesis. *Nat Genet*. 2012 Nov;44(11):1179-81. PMCID: PMC3483435.
67. Kämpjärvi K, Mäkinen N, Kilpivaara O, Arola J, Heinonen HR, Böhm J, **Abdel-Wahab O**, Lehtonen HJ, Pelttari LM, Mehine M, Schrewe H, Nevanlinna H, Levine RL, Hokland P, Böhling T, Mecklin JP, Bützow R, Aaltonen LA, Vahteristo P. Somatic MED12 mutations in uterine leiomyosarcoma and colorectal cancer. *Br J Cancer*. 2012 Nov 6;107(10):1761-5. PMID: 23132392.
68. **Abdel-Wahab O**, Adli M, Lafave LM, Gao J, Hricik T, Shih AH, Pandey S, Patel JP, Chung YR, Koche R, Perna F, Zhao X, Taylor JE, Park CY, Carroll M, Melnick A, Nimer SD, Jaffe JD, Aifantis I, Bernstein BE, Levine RL. ASXL1 Mutations Promote Myeloid Transformation through Loss of PRC2-Mediated Gene Repression. *Cancer Cell*. 2012 Aug 14;22(2):180-93. PMCID: PMC3422511.
69. Dey A, Seshasayee D, Noubade R, French DM, Liu J, Chaurushiya MS, Kirkpatrick DS, Pham VC, Lill JR, Bakalarski CE, Wu J, Phu L, Katavolos P, LaFave LM, **Abdel-Wahab O**, Modrusan Z, Seshagiri S, Dong K, Lin Z, Balazs M, Suriben R, Newton K, Hymowitz S, Garcia-Manero G, Martin F, Levine RL, Dixit VM. Loss of the tumor suppressor BAP1 causes myeloid transformation. *Science*. 2012 Sep 21;337(6101):1541-6. PMID:22878500.
70. Bejar R, Stevenson KE, Caughey BA, **Abdel-Wahab O**, Steensma DP, Galili N, Raza A, Kantarjian H, Levine RL, Neuberg D, Garcia-Manero G, Ebert BL. Validation of a prognostic model and the impact of mutations in patients with lower-risk myelodysplastic syndromes. *J Clin Oncol.* 2012 Sep 20;30(27):3376-82. PMID:22869879.
71. Gönen M, Sun Z, Figueroa ME, Patel JP, **Abdel-Wahab O**, Racevskis J, Ketterling RP, Fernandez H, Rowe JM, Tallman MS, Melnick A, Levine RL, Paietta E. CD25 expression status improves prognostic risk classification in AML independent of established biomarkers: ECOG phase 3 trial, E1900. *Blood*. 2012 Sep 13;120(11):2297-306. PMID: 22855599.
72. Koppikar P, Bhagwat N, Kilpivaara O, Manshouri T, Adli M, Hricik T, Liu F, Saunders LM, Mullally A, **Abdel-Wahab O**, Leung L, Weinstein A, Marubayashi S, Goel A, Gönen M, Estrov Z, Ebert BL, Chiosis G, Nimer SD, Bernstein BE, Verstovsek S, Levine RL. Heterodimeric JAK-STAT activation as a mechanism of persistence to JAK2 inhibitor therapy. *Nature*. 2012 Sep 6;489(7414):155-9. PMID:22820254.
73. Pollyea DA, Kohrt HE, Zhang B, Zehnder J, Schenkein D, Fantin V, Straley K, Vasanthakumar A, **Abdel-Wahab O**, Levine R, Godley LA, Medeiros BC. 2-Hydroxyglutarate in IDH Mutant AML: Predicting Patient Responses, Minimal Residual Disease and Correlations with

Methylcytosine and Hydroxymethylcytosine Levels. *Leuk Lymphoma*. 2012 Jun 11. PMID: 22680765.

74. Zhang SJ, Rampal R, Manshouri T, Patel J, Mensah N, Kayserian A, Hricik T, Heguy A, Hedvat C, Gönen M, Kantarjian H, Levine RL, **Abdel-Wahab O (co-corresponding author)**, Verstovsek S. Genetic analysis of patients with leukemic transformation of myeloproliferative neoplasms shows recurrent SRSF2 mutations that are associated with adverse outcome. *Blood.* 2012; 119(19):4480-5. PMCID:PMC3362363.
75. Patel, JP, Gonen, M, Figueroa, M, Fernandez, HF, Zhuoxin, S, Van Vlierberghe, P, Dolgalev, I, Thomas, S, Aminova, O, Huberman, K, Cheng, J, Viale, A, Socci, ND, Heguy, A, Cherry, A, Vance, G, Higgins, R, Ketterling, R, Gallagher, R, Litzow, M, van den Brink, M, Lazarus, H, Rowe, J, Luger, S, Ferrando, AF, Paietta, E, Tallman, MS, Melnick, AM, **Abdel-Wahab, O (co-last author)**, and Levine, RL. Prognostic and therapeutic relevance of integrated genetic profiling in AML. *N Engl J Med.* 2012; 22; 366(12):1079-89. PMID:22417203.
76. Lu, C, Ward, PS, Kapoor, GS, Rohle, D, Turcan, S, **Abdel-Wahab, O**, Edwards, CR, Khanin, R, Figueroa, ME, Melnick, A, Wellen, KE, O'Rourke, DM, Berger, SL, Chan, TA, Levine, RL, Mellinghoff, IK, Thompson, CB. IDH mutation impairs histone demethylation and results in a block to cell differentiation. *Nature* 2012; 15;483(7390):474-8. PMID: 22343901.
77. Dawson, MA, Bannister, AJ, Saunders, L, **Abdel-Wahab, O**, Liu, F, Nimer, SD, Levine, RL, Göttgens, B, Kouzarides, T, and Green, AR. Nuclear JAK2. *Blood* 2011; 22; 118:6987-8. PMID:22194397.
78. Betts, BC, **Abdel-Wahab O**, Curran SA, St Angelo ET, Koppikar P, Heller G, Levine RL, Young JW. Janus kinase-2 inhibition induces durable tolerance to alloantigen by human dendritic cell-stimulated T cells, yet preserves immunity to recall antigen. *Blood.* 2011 Nov 10;118(19):5330-9. PMCID:PMC3217413.
79. Ward, P, Cross, J, Lu, C, Weigert, O, **Abdel-Wahab, O**, Levine, R, Weinstock, D, Sharp, K, and Thompson, C. Identification of additional IDH mutations associated with oncometabolite R(-)-2-hydroxyglutarate production. *Oncogene.* 2012 May 10;31(19):2491-8. PMCID: PMC3271133.
80. Moran-Crusio, K, Reavie, L (co-first author), Shih, A (co-first author), **Abdel-Wahab, O**, Ndiaye-Lobry, D, Lobry, C, Figueroa, ME, Vasanthakumar, A, Patel, J, Zhao, X, Perna, F, Pandey, S, Madzo, J, Song, C, Dai, Q, He, C, Ibrahim, S, Beran, M, Zavadil, J, Nimer, SD, Melnick, A, Godley, LA, Aifantis, I, and Levine RL. *Tet2* loss leads to increased hematopoietic stem cell self-renewal and myeloid transformation. *Cancer Cell,* 2011 Jul 12;20(1):11-24. PMCID:PMC3194039.
81. Payne, EM, Bolli, N, Rhodes, J, **Abdel-Wahab, O**, Levine, RL, Hedvat, C, Stone, R, Khanna-Gupta, R, Sun, H, Kanki, J, Gazda, HT, Beggs, AH, Cotter, F, and Look, AT. Ddx18 is essential for cell cycle progression in zebrafish hematopoietic cells and is mutated in human acute myeloid leukemia. *Blood* 2011 Jul 28. 118(4):903-15. PMCID:PMC3148170.
82. Bejar, R, Stevenson, K, **Abdel-Wahab, O**, Galili, N, Nilsson, B, Garcia-Manero, G, Kantarjian, H, Raza, A, Levine, RL, Neuberg, D, and Ebert, B. Mutations in Myelodysplastic Syndromes Are Independent Predictors of Overall Survival and Are Associated With Clinical Features. *New England J Medicine* 2011 Jun 30;364(26):2496-506. PMCID:PMC3159042
83. Klinakis A, Lobry C, **Abdel-Wahab O**, Oh P, Haeno H, Buonamici S, van De Walle I, Cathelin S, Trimarchi T, Araldi E, Liu C, Ibrahim S, Beran M, Zavadil J, Efstratiadis A, Taghon T, Michor F, Levine RL, Aifantis I. A novel tumour-suppressor function for the Notch pathway in myeloid leukaemia. *Nature*. 2011 May 12;473(7346):230-3. PMID: 21562564
84. Zhou L, Opalinska J, Sohal D, Yu Y, Mo Y, Bhagat T, **Abdel-Wahab O**, Fazzari M, Figueroa M, Alencar C, Zhang J, Kanbhampati S, Parmar S, Nischal S, Heuck C, Suzuki M, Friedman E, Pellagatti A, Boultwood J, Steidl U, Sauthararajah Y, Yajnik V, McMahon C, Gore SD, Platanias LC, Levine R, Melnick A, Wickrema A, Greally JM, Verma A. Aberrant epigenetic and genetic marks are seen in myelodysplastic leucocytes and reveal DOCK4 as a candidate pathogenic gene on chr7q. *J Biol Chem* 2011 Jul 15;286(28):25211-23. PMCID:PMC3137092
85. **Abdel-Wahab, O,** Pardanani, A, Patel, J, Wadleigh, M, Lasho, T, Heguy, A, Beran, M, Gililland, DG, Levine, R, and Tefferi, A. Concomitant analysis of ASXL1 and EZH2 mutations in myelofibrosis, chronic myelomonocytic leukemia, and blast-phase myeloproliferative neoplasms. *Leukemia* 2011 Jul;25(7):1200-2. PMID:21455215

86. Perna, F, **Abdel-Wahab, O**, Levine, RL, Jhanwar, SC, Imada, K, Nimer, SD. ETV6-ABL1-positive "chronic myeloid leukemia": clinical and molecular response to tyrosine kinase inhibition. *Haematologica* 2011 Feb;96(2):342-3. PMCID: PMC3031707.
87. Liu, F, Zhao, X (co-first author), Perna, F, Wang, L, Koppikar, P, **Abdel-Wahab, O**, Harr, M, Levine, RL, Xu, H, Tefferi, A, Hatlen, M, Menendez, S, Nimer. SD. JAK2V617F-mediated phosphorylation of PRMT5 down-regulates its methyltransferase activity and promotes myeloproliferation. *Cancer Cell* 2011 Feb 15;19(2):283-94. PMID:21316606.
88. Figueroa ME, **Abdel-Wahab O (Co-first author),** Lu C (Co-first author), Ward PS, Patel J, Shih A, Li Y, Bhagwat N, Vasanthakumar A, Fernandez HF, Tallman MS, Sun Z, Wolniak K, Peeters JK, Liu W, Choe SE, Fantin VR, Paietta E, Löwenberg B, Licht JD, Godley LA, Delwel R, Valk PJ, Thompson CB, Levine RL, Melnick A. Leukemic IDH1 and IDH2 Mutations Result in a Hypermethylation Phenotype, Disrupt TET2 Function, and Impair Hematopoietic Differentiation. *Cancer Cell.* 2010 18(6) pp. 553 – 567. PMID:21130701.
89. Van Vlierberghe, P, Patel, J, **Abdel-Wahab, O**, Lobry, C, Hedvat, CV, Balbin, M, Nicolas, C, Ramirez Payer, A, Fernandez, HF, Tallman, MS, Paietta, E, Melnick, A, Vandenberghe, P, Speleman, F, Aifantis, I, Cools, J, Levine, R, and Ferrando, A. *PHF6* mutations in adult acute myeloid leukemia. *Leukemia*. 2011 Jan;25(1):130-4. PMID:21030981.
90. Attolini CS, Cheng YK, Beroukhim R, Getz G, **Abdel-Wahab O**, Levine RL, Mellinghoff IK, Michor F. A mathematical framework to determine the temporal sequence of somatic genetic events in cancer. *Proc Natl Acad Sci U S A.* 2010 Oct 12;107(41):17604-9. PMCID:PMC2955151.
91. Marubayashi S, Koppikar P, Taldone T, **Abdel-Wahab O**, West N, Bhagwat N, Caldas-Lopes E, Ross KN, Gönen M, Gozman A, Ahn JH, Rodina A, Ouerfelli O, Yang G, Hedvat C, Bradner JE, Chiosis G, Levine RL. HSP90 is a therapeutic target in JAK2-dependent myeloproliferative neoplasms in mice and humans*. J Clin Invest*. 2010 Oct;120(10):3578-93.PMCID:PMC2947224.
92. Espinosa L, Cathelin S, D'Altri T, Trimarchi T, Statnikov A, Guiu J, Rodilla V, Inglés-Esteve J, Nomdedeu J, Bellosillo B, Besses C, **Abdel-Wahab O**, Kucine N, Sun SC, Song G, Mullighan CC, Levine RL, Rajewsky K, Aifantis I, Bigas A. The Notch/Hes1 pathway sustains NF-κB activation through CYLD repression in T cell leukemia. *Cancer Cell.* 2010 Sep 14;18(3):268-81. PMCID:PMC2963042.
93. Perna, F, Gurvich, N, Hoya-Arias, R, **Abdel-Wahab, O**, Levine, RL, Asai, T, Voza, F, Menendez, S, Wang, L, Liu, F, Zhao, X, Nimer, SD. Depletion of L3MBTL1 promotes the erythroid differentiation of human hematopoietic progenitor cells: possible role in 20q- polycythemia vera. *Blood.* 2010 Oct 14;116(15):2812-21.PMCID:PMC2974589
94. **Abdel-Wahab, O**, Kilpivaara, O, Patel, J, Busque, L, Levine, RL. The most commonly reported variant in *ASXL1* (c.1934dupG;p.Gly646TrpfsX12) is not a somatic alteration. *Leukemia* 2010 Sep;24(9):1656-7. PMID:20596031.
95. Tefferi, A, Lasho, TL, **Abdel-Wahab, O**, Guglielmelli, P, Patel, J, Caramazza, D, Pieri, L, Finke, CM, Kilpivaara, O, Wadleigh, M, Mai, M, McClure, RF, Gilliland, DG, Levine, RL, Pardanani, A, Vannucchi, AM. *IDH1* and *IDH2* mutation studies in 1,473 patients with chronic-, fibrotic- or blast-phase essential thrombocythemia, polycythemia vera or myelofibrosis. *Leukemia* 2010 Jul;24(7):1302-9. PMCID:PMC3035975.
96. Ward PS, Patel J, Wise DR, **Abdel-Wahab O**, Bennett BD, Coller HA, Cross JR, Fantin VR, Hedvat CV, Perl AE, Rabinowitz JD, Carroll M, Su SM, Sharp KA, Levine RL, Thompson CB. The common feature of leukemia-associated IDH1 and IDH2 mutations is a neomorphic enzyme activity converting alpha-ketoglutarate to 2-hydroxyglutarate. *Cancer Cell.* 2010 Mar 16;17(3):225-34. PMCID:PMC2849316.
97. Koppikar, P, **Abdel-Wahab, O**, **(co-first author),** Hedvat, C, Marubayashi, S, Patel, J, Goel, A, Kucine, N, Gardner, J, Combs, AP, Vaddi, K, Haley, PJ, Burn, TC, Rupar, M, Bromberg, JF, Heaney, ML, de Stanchina, E, Fridman, JS, Levine, RL. Efficacy of the JAK2 Inhibitor INCB16562 in a Murine Model of MPLW515L-Induced Thombocytosis and Myelofibrosis. *Blood*. 2010 Apr 8;115(14):2919-27. PMCID:PMC2854434.
98. **Abdel-Wahab O**, Manshouri T, Patel J, Harris K, Yao J , Hedvat C , Heguy A, Bueso-Ramos C, Kantarjian H, Levine RL, Verstovsek S. Genetic analysis of leukemic transformation of chronic myeloproliferative neoplasms. *Canc Res* 2010 Jan 15;70(2):447-52. PMCID:PMC2947340.

99. Hussein K, **Abdel-Wahab O**, Lasho TL, Van Dyke DL, Levine RL , Hanson CA, Pardanani A, Tefferi A. Cytogenetic correlates of *TET2* mutations in 199 patients with myeloproliferative neoplasms. *Am J Hematol* 2010 Jan;85(1):81-3. PMID:19957346.
100. **Abdel-Wahab O**, Mullally A, Hedvat C, Garcia-Manero G, Patel J, Wadleigh M, Malinge S, Yao J, Kilpivaara O, Bhat R, Huberman K, Thomas S, Dolgalev I, Heguy A, Paietta E, Le Beau MM, Beran M, Tallman MS, Ebert BL, Kantarjian HM, Stone RM, Gilliland DG, Crispino JD, Levine RL. Genetic characterization of TET1, TET2, and TET3 alterations in myeloid malignancies. *Blood.* 2009 Jul 2;114(1):144-7. PMCID:PMC2710942.
101. Tefferi A, Lim KH, **Abdel-Wahab O**, Lasho TL, Patel J, Patnaik MM, Hanson CA, Pardanani A, Gilliland DG, Levine RL. Detection of mutant TET2 in myeloid malignancies other than myeloproliferative neoplasms: CMML, MDS, MDS/MPN and AML. *Leukemia.* 2009 Jul;23(7):1343-5. PMID:19295549.
102. Tefferi, A, Pardanani, A, Lim, K-H, **Abdel-Wahab, O**, Lasho, TL, Patel, J, Gangat, N, Finke, Schwager, S, Mullally, A, Li, C-Y, Hanson, CA, Mesa, R, Bernard, O, Delhommeau, F, Vainchenker, W, Gilliland, DG, and Levine, RL. TET2 mutations and their clinical correlates in polycythemia vera, essential thrombocythemia and myelofibrosis. *Leukemia*. 2009 May;23(5):905-11. PMID:19262601.
103. Tefferi, A, Levine, RL, Lim, K-H, **Abdel-Wahab, O**, Lasho, TL, Patel, J, Finke, CM, Mullally, A, Li, C-Y, Pardanani, A and Gilliland, DG. Frequent TET2 mutations in systemic mastocytosis: clinical, KITD816V and FIP1L1-PDGFRA correlates. *Leukemia.* 2009 May;23(5):900-4. PMID:19262601.
104. **Abdel-Wahab, OI**, Rosovsky, RP, Warth, JA. Warfarin-induced skin necrosis in a patient with heparin-induced thrombocytopenia: two diseases or one? *Acta Haematol.* 2008;120(2):117-22 PMID:19018128.
105. Ueno T, de la Fuente SG, **Abdel-Wahab OI**, Takahashi T, Gottfried M, Harris MB, Tatewaki M, Uemura K, Lawson DC, Mantyh CR, Pappas TN. Functional evaluation of the grafted wall with porcine-derived small intestinal submucosa (SIS) to a stomach defect in rats. *Surgery.* 2007 142(3): 376-83. PMID:17723890.
106. Darabi, K, **Abdel-Wahab, O**, Stowell, C., Sepher, A. An 87-year-old woman with respiratory distress and alveolar hemorrhage after transfusion. *Chest 2006* Nov;130(5):1612-6. PMID:17099045.
107. **Abdel-Wahab, OI**, Healy B, Dzik, WH. Effect of FFP transfusion on prothrombin time and risk of bleeding in patients with mild coagulation abnormalities. *Transfusion* 2006 Aug;46(8):1279-85. PMID:16934060.
108. Darabi, K, **Abdel-Wahab, O**, Dzik, WH. Current usage of intravenous immune globulin and the rationale behind it: the Massachusetts General Hospital data and a review of the literature. *Transfusion* 2006 May;46(5):741-53. PMID:16686841.
109. Ueno T, Ko SH, Grubbs E, Yoshimoto Y, Augustine C, Abdel-Wahab Z, Cheng TY, **Abdel-Wahab OI**, Pruitt SK, Friedman HS, Tyler DS. Modulation of chemotherapy resistance in regional therapy: a novel therapeutic approach to advanced extremity melanoma using intra-arterial temozolomide in combination with systemic O6-benzylguanine. *Mol Cancer Ther* 2006 Mar;5(3):732-8. PMID:16546988.
110. Ko, SH, Ueno, T, Yoshimoto, Y, Yoo, JS, **Abdel-Wahab, OI**, Abdel-Wahab, Z, Chu, E, Pruitt, SK, Friedman, HS, Dewhirst, MW, Tyler, DS. Optimizing a novel regional chemotherapeutic agent against melanoma: hyperthermia-induced enhancement of temozolimide cytotoxicity. *Clinical Canc Res* 2006 Jan 1;12(1):289-97. PMID:16397054.
111. Abdel-Wahab, Z, Cisco, R, Dannull, J, Ueno, T, **Abdel-Wahab, O**, Kalady, MF, Onaitis, M, Tyler, DS, Pruitt, SK. Cotransfection of DC with TLR4 and MART-1 RNA induces MART-1-specific responses. *J Surg Res* 2005 Apr;124(2):264-73. PMID:15820257.
112. Kalady, MF, Peterson, B, Baillie, J, Onaitis, MW, **Abdel-Wahab, OI**, Howden, JK, Jowell, PS, Branch, S, Clary, BM, Pappas, TN, Tyler, DS. Pancreatic duct strictures: identifying risk of malignancy. *Ann Surg Oncol* 2004 Jun;11(6):581-8. PMID:15150064.
113. **Abdel-Wahab, OI**, Grubbs, E, Viglianti, B, Cheng, T, Ueno, T, Ko, S, Rabbani, Z, Curtis, S, Pruitt, SK, Dewhirst, MW, Tyler, DS. Role of hyperthermia in regional alkylating agent chemotherapy. *Clin Can Res 2004* Sep 1;10(17):5919-29. PMID:15355925.

114. Cheng TY, Grubbs E, **Abdul-Wahab O**, Leu SY, Hung CF, Petros W, Aloia T, Fedrau R, Pruitt SK, Colvin M, Friedman H, and Tyler DS. Marked variability of melphalan drug levels during regional hyperthermic isolated limb perfusion. *Am J Surg* 2003 Nov;186(5):460-7. PMID:14599607.
115. Abdel-Wahab, Z, Kalady, M, Emani, S, Onaitis, M, **Abdel-Wahab, O**, Cisco, R, Wheeless, L, Yen-Cheng, T, Tyler, D, Pruitt, S. Dendritic cells transfected with RNA encoding mutated full-length Melan-A/Mart-1 with an A27L amino-acid substitution induce a potent anti-melanoma CTL response. *Cell Immunol* 2003 Aug;224(2):86-97. PMID:14609574.

Books, Book Chapters and Reviews

1. Joshi, P, Halene, S, **Abdel-Wahab, O**. How do mRNA splicing alterations drive myelodysplasia? *Blood* 2017 (in press)
2. Taylor, J, and **Abdel-Wahab, O**. Stem Cell Model of Disease. In Hematologic Diseases in Hematology, Basic Principles and Practice, Seventh Edition. Elsevier
3. Lee, SCW and **Abdel-Wahab, O**. Modeling CBL activating mutations in vivo. *Blood* 2017 (in press)
4. Liu, B and **Abdel-Wahab, O**. Partial loss of genes might open therapeutic window. *ELife* 2017 Mar 17;6. pii: e25996. doi: 10.7554/eLife.25996. PubMed PMID: 28304277; PubMed Central PMCID: PMC5357136.
5. Haroche, J, Cohen-Aubart, F, Rollins, B, Donadieu, J, Charlotte, F, Idbaih, A, Vaglio, A, **Abdel-Wahab, O,** Emile, J-F, Amoura, Z. Histiocytoses: emerging neoplasia behind inflammation. *Lancet Oncol* 2017 Feb;18(2):e113-e125. doi: 10.1016/S1470-2045(17)30031-1. Review. PubMed PMID: 28214412.
6. Yoshimi A, **Abdel-Wahab O**. Molecular Pathways: Understanding and Targeting Mutant Spliceosomal Proteins. *Clin Cancer Res*. 2017 Jan 15;23(2):336-341. doi: 10.1158/1078-0432.CCR-16-0131. PubMed PMID: 27836865; PubMed Central PMCID: PMC5241248.
7. Grever MR, Abdel-Wahab O, Andritsos LA, Banerji V, Barrientos J, Blachly JS, Call TG, Catovsky D, Dearden C, Demeter J, Else M, Forconi F, Gozzetti A, Ho AD, Johnston JB, Jones J, Juliusson G, Kraut E, Kreitman RJ, Larratt L, Lauria F, Lozanski G, Montserrat E, Parikh SA, Park JH, Polliack A, Quest GR, Rai KR, Ravandi F, Robak T, Saven A, Seymour JF, Tadmor T, Tallman MS, Tam C, Tiacci E, Troussard X, Zent CS, Zenz T, Zinzani PL, Falini B. Consensus guidelines for the diagnosis and management of patients with classic hairy cell leukemia. Blood. 2017 Feb 2;129(5):553-560. doi: 10.1182/blood-2016-01-689422. Review. PubMed PMID: 27903528.
8. Schuettpelz, L, **Abdel-Wahab, O**. Molecular Basis of Hematology. In American Society of Hematology Self-Assessment Program, 6th edition. Washington, DC: American Society of Hematology (ASH).
9. Inoue, D, **Abdel-Wahab, O**. Modeling SF3B1 Mutations in Cancer: Advances, Challenges, and Opportunities. Cancer Cell. 2016 Sep 12;30(3):371-3. doi: 10.1016/j.ccell.2016.08.013. PubMed PMID: 27622329.
10. Yoshimi A, **Abdel-Wahab, O**. Defining risk in MDS over time. Blood. 2016 Aug 18;128(7):885-6. doi: 10.1182/blood-2016-07-724930. PubMed PMID: 27539994.
11. Lee SC, **Abdel-Wahab, O**. Therapeutic targeting of splicing in cancer. Nat Med. 2016 Sep 7;22(9):976-86. doi: 10.1038/nm.4165. Review. PubMed PMID: 27603132.
12. Micol JB, **Abdel-Wahab, O**. The Role of Additional Sex Combs-Like Proteins in Cancer. Cold Spring Harb Perspect Med. 2016 Aug 15. pii: a026526. doi: 10.1101/cshperspect.a026526. [Epub ahead of print] PubMed PMID: 27527698.
13. Ntziachristos P, **Abdel-Wahab O**, Aifantis I. Emerging concepts of epigenetic dysregulation in hematological malignancies. Nat Immunol. 2016 Aug 1. doi: 10.1038/ni.3517. [Epub ahead of print] Review. PubMed PMID: 27478938.
14. Dvinge H, Kim E, **Abdel-Wahab O (co-corresponding),** Bradley RK. RNA splicing factors as oncoproteins and tumour suppressors. Nat Rev Cancer. 2016 Jun 10. doi: 10.1038/nrc.2016.51. [Epub ahead of print] PubMed PMID: 27282250.

15. Mughal, TI, **Abdel-Wahab, O**, Rampal, R, Mesa, R, Koschmieder, S, Levine, R, Hehlmann, R, Saglio, G, Barbui, T, Van Etten, RA. Contemporary insights into the pathogenesis and treatment of chronic myeloproliferative neoplasms. Leuk Lymphoma. 2016 May 31:1-10. [Epub ahead of print] PubMed PMID: 27240645.
16. Inoue D, Bradley RK, **Abdel-Wahab O**. Spliceosomal gene mutations in myelodysplasia: molecular links to clonal abnormalities of hematopoiesis. Genes Dev. 2016 May 1;30(9):989-1001. doi: 10.1101/gad.278424.116. Review. PubMed PMID: 27151974; PubMed Central PMCID: PMC4863743.
17. Durham BH, Diamond EL, **Abdel-Wahab O**. Histiocytic neoplasms in the era of personalized genomic medicine. Curr Opin Hematol. 2016 Apr 20. [Epub ahead of print] PubMed PMID: 27101528.
18. Emile JF, Abla O, Fraitag S, Horne A, Haroche J, Donadieu J, Requena-Caballero L, Jordan MB, **Abdel-Wahab O**, Allen CE, Charlotte F, Diamond EL, Egeler RM, Fischer A, Gil Herrera J, Henter JI, Janku F, Merad M, Picarsic J, Rodriguez-Galindo C, Rollins BJ, Tazi A, Vassallo R, Weiss LM. Revised classification of histiocytoses and neoplasms of the macrophage-dendritic cell lineages. Blood. 2016 Mar 10. pii: blood-2016-01-690636. [Epub ahead of print] PubMed PMID: 26966089.
19. Salam L, **Abdel-Wahab O**. Hairy cell leukemia: update and current therapeutic approach. *Curr Opin Hematol.* 2015 Jul;22(4):355-61. doi: 10.1097/MOH.0000000000000154. PubMed PMID: 26049757.
20. Mughal TI, Barbui T, **Abdel-Wahab O**, Kralovics R, Jamieson C, Kvasnicka HM, Mullaly A, Rampal R, Mesa R, Kiladjian JJ, Deininger M, Prchal J, Hehlmann R, Saglio G, Van Etten RA. Novel Insights into the Biology and Treatment of Chronic Myeloproliferative Neoplasms. Leuk Lymphoma. 2014 Oct 20:1-19. [Epub ahead of print] PubMed PMID: 25330439.
21. Lee, S. C.-W., Abdel-Wahab, O. The mutational landscape of paroxysmal nocturnal hemoglobinuria revealed: new insights into clonal dominance. *J Clin Invest.* 2014 Oct;124(10):4227-30. doi: 10.1172/JCI77984. PubMed PMID: 25244089; PubMed Central PMCID: PMC4191026.
22. Abdel-Wahab, O, and Park, CY. *BRAF*-mutant hematopoietic malignancies. *Oncotarget* 2014 Sep 30;5(18):7980-1. PubMed PMID: 25478626; PubMed Central PMCID: PMC4226659.
23. Aumann S, **Abdel-Wahab O**. Somatic alterations and dysregulation of epigenetic modifiers in cancers. *Biochem Biophys Res Commun*. 2014 [Epub ahead of print] Review. PubMed PMID: 25111821.
24. Chung, Y. R., Kim, E., **Abdel-Wahab, O**. Femoral Bone Marrow Aspiration in Live Mice. *J. Vis. Exp.* 2014 July 5. PMID: 25045847
25. Diamond, EL, Dagna, L, Hyman, DM, Cavalli, G, Janku, F, Estrada-Veras, J, **Abdel-Wahab, O**, Heaney, ML, Scheel, PJ, Feeley, NK, Ferrero, E, McClain, KL, Vaglio, A, Colby, T, Arnaud, L, Haroche, J. Consensus Guidelines for the Diagnosis and Clinical Management of Erdheim-Chester Disease. *Blood* 2014. 2014 July 24. PMID: 24850756 PMCID: PMC4110656.
26. Mughal TI, Vannucchi AM, Soverini S, Bazeos A, Tibes R, Saglio G, **Abdel-Wahab O,** Pardanani A, Hehlmann R, Barbui T, Van Etten R, Tefferi A, Goldman JM. Current pre-clinical and clinical advances in the BCR-ABL1-positive and -negative chronic myeloproliferative neoplasms. Haematologica. 2014 May;99(5):797-801. PubMed PMID: 24790057.
27. Khasawneh MK, **Abdel-Wahab O**. Recent Discoveries in Molecular Characterization of Acute Myeloid Leukemia. Curr Hematol Malig Rep. 2014 Mar 9. [Epub ahead of print] PubMed PMID: 24609756.
28. Glass, J. L. and **Abdel-Wahab, O** (2014). Epigenetic Testing. In *Pocket Oncology* (pp. 17-32). New York, NY: Lippincott Williams & Wilkins.
29. Micol JB, Abdel-Wahab O. Collaborating constitutive and somatic genetic events in myeloid malignancies: ASXL1 mutations in patients with germline GATA2 mutations. Haematologica. 2014 Feb;99(2):201-3. doi: 10.3324/haematol.2013.101303. PubMed PMID: 24497555; PubMed Central PMCID: PMC3912947.
30. Kim E, **Abdel-Wahab O.** Focus on the epigenome in the myeloproliferative neoplasms. Hematology Am Soc Hematol Educ Program. 2013;2013:538-44.
31. Bejar, R, **Abdel-Wahab, O.** The importance of subclonal genetic events in MDS. *Blood* 2013; 122:3550-3551

32. Mughal TI, Girnius S, Rosen ST, Kumar S, Wiestner A, **Abdel-Wahab O**, Kiladjian JJ, Wilson WH, Van Etten RA. Emerging therapeutic paradigms to target the dysregulated JAK/STAT pathways in hematologicalmalignancies. Leuk Lymphoma. 2013 Nov 11. [Epub ahead of print] PubMed PMID: 24206094.
33. Padron E, **Abdel-Wahab O.** Importance of Genetics in the Clinical Management of Chronic Myelomonocytic Leukemia. J Clin Oncol. 2013 May 20. [Epub ahead of print] PubMed PMID: 23690427.
34. **Abdel-Wahab O**, Levine RL. Mutations in epigenetic modifiers in the pathogenesis and therapy of acute myeloid leukemia. Blood. 2013 May 2;121(18):3563-72. doi: 10.1182/blood-2013-01-451781. Review. PubMed PMID: 23640996; PubMed Central PMCID: PMC3643757.
35. Bhatt PK, **Abdel-Wahab O**. Refining the prognostic importance of the diversity of FLT3 internal tandem duplications. *Leuk Lymphoma* 2012. 2013 Jan;54(1):3-4. PMID:22775313.
36. **Abdel-Wahab O**, Figueroa ME. Interpreting new molecular genetics in myelodysplastic syndromes. *Hematology Am Soc Hematol Educ Program*. 2012;2012:56-64. doi: 10.1182/asheducation-2012.1.56. PMID: 23233561.
37. Mascarenhas J, Heaney ML, Najfeld V, Hexner E, **Abdel-Wahab O**, Rampal R, Ravandi F, Petersen B, Roboz G, Feldman E, Podoltsev N, Douer D, Levine R, Tallman M, Hoffman R. Proposed criteria for response assessment in patients treated in clinical trials for myeloproliferative neoplasms in blast phase (MPN-BP): Formal recommendations from the post-myeloproliferative neoplasm acute myeloid leukemia consortium. *Leuk Res.* 2012 Dec;36(12):1500-4.PMID:22938832.
38. Chung YR, Schatoff E, **Abdel-Wahab O**. **(co-first author).** Epigenetic alterations in hematopoietic malignancies. *Int J Hematol*. 2012 Oct;96(4):413-27. PMID:23015417.
39. **Abdel-Wahab O**, Tefferi A, Levine RL. Role of TET2 and ASXL1 mutations in the pathogenesis of myeloproliferative neoplasms. *Hematol Oncol Clin North Am.* 2012 Oct;26(5):1053-64. PMCID:PMC3459179.
40. Shih AH, **Abdel-Wahab O**, Patel JP, Levine R.L., The role of mutations in epigenetic regulators in myeloid malignancies. *Nat Rev Cancer*. 2012 Aug 14;22(2):180-93. PMCID:PMC3422511.
41. **Abdel-Wahab O**. Molecular genetics of acute myeloid leukemia: clinical implications and opportunities for integrating genomics into clinical practice. *Hematology.* 2012 Apr;17 Suppl 1:S39-42. PMID:22507776.
42. **Abdel-Wahab, O**, Pardanani, A, Bernard, OA, Finazzi, G, Crispino, JD, Gisslinger, H, Kralovics, R, Odenike, O, Bhalla, K, Gupta, V, Barosi, G, Gotlib, J, Guglielmelli, P, Kiladjian, J-J, Noel, P, Cazzola, M, Vannucchi, AM, Hoffman, R, Barbui, T, Thiele, J, Van Etten, RA, Mughal, T, Tefferi, A. Unraveling the genetic underpinnings of myeloproliferative neoplasms and understanding their effect on disease course and response to therapy: proceedings from the 6th International Post-ASH Symposium. *Am J Hematol* 2012 May;87(5):562-8. PMCID:PMC3491640.
43. Sadrzadeh H, **Abdel-Wahab O**, Fathi AT. Molecular alterations underlying eosinophilic and mast cell malignancies. *Discov Med.* 2011 Dec;12(67):481-93. PMID:22204765.
44. Zhang SJ, **Abdel-Wahab O**. Disordered Epigenetic Regulation in the Pathophysiology of Myeloproliferative Neoplasms. *Curr Hematol Malig Rep*. 2012 Mar;7(1):34-42. PMID: PMID:22170482.
45. **Abdel-Wahab O**, Patel J, Levine RL. Clinical Implications of Novel Mutations in Epigenetic Modifiers in AML. *Hematol Oncol Clin North Am.* 2011 Dec;25(6):1119-33. PMID:22093580.
46. **Abdel-Wahab O,** Levine R. The spliceosome as an indicted conspirator in myeloid malignancies. *Cancer Cell*. 2011 Oct 18;20(4):420-3. PMCID:PMC3218079.
47. Cimmino, L, **Abdel-Wahab, O**, Levine, RL, and Aifantis, I. The regulation of DNA methylation by TET family proteins and their role in hematopoietic stem cell differentiation and transformation. *Cell Stem Cell* 2011 Sep 2;9(3):193-204. PMCID:PMC3244690.
48. Soriano Pignataro, D, **Abdel-Wahab, O.** FISHing for TET2: utility of FISH for TET2 deletion detection in clinical samples. *Leukemia Research* 2012 Jan;36(1):25-6. PMID:21917313.
49. Fathi, A, **Abdel-Wahab, O.** Mutations in epigenetic modifiers in myeloid malignancies and the prospect of novel epigenetic-targeted therapy. *Adv Hematol,* 2012;2012:469592; PMCID:PMC3145345.
50. Tefferi, A, **Abdel-Wahab, O**, Cervantes, F, Crispino, JD, Finazzi, G, Girodon, F, Gisslinger, H, Gotlib, J, Kiladjian, J-J, Levine, RL, Licht, JD, Mullally, A, Odenike, O, Pardanani, A, Silverm RT,

Solary, E, and Mughal, T. Unraveling the genetic underpinnings of myeloproliferative neoplasms and understanding their effect on disease course and response to therapy: proceedings from the 6th International Post-ASH Symposium. *Am. J Hematology.* 2012 May;87(5):562-8. PMCID:PMC3491640.
51. **Abdel-Wahab O.** Genetics of the myeloproliferative neoplasms. *Curr Opin Hematol* 2011 Mar;18(2):117-23. PMID:21307773.
52. **Abdel-Wahab O,** Levine RL. Recent advances in the treatment of acute myeloid leukemia. *F1000 Med Rep.* 2010 Jul 22;2:55. PMCID:PMC2927833.
53. **Abdel-Wahab, O** and Levine, RL. *EZH2* mutations: mutating the epigenetic machinery in myeloid malignancies. *Cancer Cell* 2010 Aug 9;18(2):105-107.
54. **Abdel-Wahab, Ol** and Levine, RL. Metabolism and the Leukemic Stem Cell. *J Exp Med* 2010 Apr 12;207(4):677-80. PMCID:PMC2856035.
55. **Abdel-Wahab, Ol** and Levine, RL. Genetics of the Myeloproliferative Neoplasms. In: Myeloproliferative Neoplasms, eds. Tefferi, A and Verstovsek, S. Springer Press 2010.
56. **Abdel-Wahab, Ol,** Levine, RL. Myelofibrosis: Myelofibrosis: An update into pathogenesis and treatment. *Annu Rev Med.* 2009;60:233-45. PMID:18947294.
57. Morse, MA, Lyerly, HK, Clay, TM, **Abdel-Wahab, O**, Chui, SY, Garst, J, Gollob, J, Grossi, PM, Kalady, M, Mosca, PJ, Onaitis, M, Sampson, JH, Seigler, HF, Toloza, EM, Tyler, D, Viewig, J, Yang, Y. Immunotherapy of surgical malignancies (review). *Curr Prob Surg* 2004 Jan;41(1):15-132. PMID:14749625.

Oral Presentations:

2009 International Conference on Differentiation Therapy, Samuel Waxman Foundation, Chicago, IL
2010 Post-ASH Myeloproliferative Neoplasm Workshop, Orlando Fla.
2011 International Working Group for Myelofibrosis Research and Treatment Workshop, Florence, Italy
2011 Leukemia Grand Rounds, Leukemia Dept, MD Anderson Cancer Center, Houston, TX
2011 Lineberger Cancer Center Grand Rounds, UNC Chapel Hill, Chapel Hill, NC
2011 FASEB Hematologic Malignancies, Saxtons River, VT
2011 Northwestern University, Hematology Grand Rounds, Chicago, IL
2011 American Society of Hematology, Oral Presentation in "Oncogenes and Tumor Suppressors"
2011 Post-ASH Myeloproliferative Neoplasm Workshop, La Jolla, CA
2012 UTSW Simmons Cancer Center Molecular Therapeutics of Cancer Program, Dallas, TX
2012 University of Pennsylvania, Dept of Cancer Biology, Philadelphia, Pennsylvania
2012 Cincinnati Children's Hospital, Experimental Hematology and Cancer Pathology Program, Cincinnati, Ohio
2012 Dept. of Hematology/Oncology, Mount Sinai College of Medicine, New York, NY
2012 Dept. of Genetics, Albert Einstein College of Medicine, Bronx, NY
2012 Chromatin Club of New York, Mount Sinai College of Medicine, New York, NY
2012 Division of Hematologic Neoplasia, Dept. of Medicine, Dana Farber Cancer Institute, Boston, MA
2012 Institut Gustave Roussy Research Seminar, INSERM, Villejuif cedex, France
2012 International Working Group for Myelofibrosis Research and Treatment Workshop, Florence, Italy
2012 Hematologic Malignancies Grand Rounds, Massachusetts General Hospital Cancer Center, Boston MA
2012 Mayo Clinic Arizona, Cancer Center Grand Rounds, Scottsdale AZ
2012 Dept. of Hematology Grand Rounds, First Affiliated Hospital of Nanjing Medical University, Nanjing, China
2012 Plenary Speaker, Chinese Society of Hematology 2012 Annual Meeting, Suzhou China
2012 XII Uruguayan Congress of Hematology, Punta Del Este Uruguay
2012 Innovation Approaches to JAK Inhibition and Continued Clinical Questions in th Management of Myelofibrosis. Atlanta, GA.

2012 American Society of Hematology, Biology of MDS Oral Session, Atlanta, GA.
2012 Post-ASH International CML and MPN Workshop, Atlanta, GA.
2013 Clinical Translation of Epigenetics in Cancer Therapy, Asheville NC
2013 St. Jude's Children's Research Hospital, Dept. Pharmaceutical Sciences Seminar Series
2013 Leukemia Lymphoma Society Panel at the Annual Cancer Progress Research Conference
2013 New York City Regional CLL Summit, Long Island Jewish Hospital
2013 International Working Group for Myelofibrosis Research and Treatment Workshop, Florence, Italy
2013 American Association of Cancer Research, Current Concepts Session "The Genetic and Epigenetic Landscape of Leukemia Revealed"
2013 Symposium of Molecular Oncology and Personalized Medicine, Albert Einstein Instituto Israelita De Ensino e Pesquisa, Sao Paulo Brazil
2013 VII Board Review Curso De Revisao Em Hematologia e Hemoterapia, Albert Einstein Instituto Israelita De Ensino e Pesquisa, Sao Paulo Brazil
2013 2013 ASCO — Clinical Problems in Oncology Session
2013 FASEB Hematologic Malignancies, Saxton's River, Vermont
2013 Society of Hematologic Oncology (SOHO) Meeting, Houston, Texas. "Epigenetic Drivers of Myelodysplasia"
2013 New York Regional CLL Summit Meeting, New York, NY
2013 Foundation Medicine, Cambridge, MA
2013 National Cancer Research Center, Tokyo Japan
2013 10th International Nikko Symposium, Utsonomiya Japan
2013 Dept. of Cell and Molecular Biology, Chiba University, Chiba Japan
2013 Hematology/Oncology Grand Rounds, Marshall University, Huntington, W. Virginia
2013 ASH Educational Session on Myeloproliferative Neoplasms
2013 ASH Oral Abstract Presentation
2013 Cold Spring Harbor Laboratories Scientific Seminar
2014 H3 Biomedicine, Inc, Cambridge MA
2014 Damon Runyon Cancer Research Foundation Accelerating Cancer Cures Symposium
2014 Aplastic Anemia & MDS Foundation International Foundation Bone Marrow Failure Disease Scientific Symposium
2014 Center for Medical Genetics, Ghent University, Ghent, Belgium
2014 The Nikolas Symposium XXIII, Athens, Greece
2014 Hairy Cell Leukemia Foundation, Houston, TX
2014 Evans Foundation MDS Summit, Philadelphia, PA
2014 Plenary Session, AACR Hematologic Sessions, Philadelphia, PA
2014 73rd Annual Meeting of the Japanese Cancer Association, Yokohama, Japan
2014 Seminar, Institute of Medical Sciences, University of Tokyo, Tokyo, Japan
2014 John Pritchard Lectureship, 30th Annual Meeting of the Histiocyte Society, Toronto, Canada
2014 "Think Tank" on Integrating New Molecular Targets in AML. Dallas, Texas
2014 Eastern Cooperative Oncology Group (ECOG) Leukemia Lab Committee. Orlando, Florida
2014 Scientific Workshop on Myeloid Development, 56th Annual Meeting of the American Society of Hematology (ASH), San Francisco, California
2014 Oral Session of Basic and Translation Studies in MDS, 56th Annual Meeting of the American Society of Hematology (ASH), San Francisco, California
2014 9th International CML and MPN post-ASH Workshop, San Francisco, California
2015 7th Biennial Workshop on "Clinical Translation of Epigenetics in Cancer Therapy", St. Augustine, Florida
2015 Plenary Speaker, Molecular Med Tri-Con, San Francisco, CA
2015 Dept. of Biochemistry Seminar, University of Virginia, Charlottesville, VA.
2015 Leukemia and Lymphoma Society Symposium on Hematological Malignancies, Northwestern University, Chicago, IL
2015 Panel on B-cell Malignancies, Cancer Progress Conference, New York, NY
2015 Research Seminar Series, Boston Children's Hospital, Boston, MA
2015 Indiana University, Wells Center for Pediatric Research Seminar Series
2015 Evans Foundation, MDS Research Summit, Washington D.C.

2015 Hairy Cell Leukemia Foundation Meeting, Chicago, IL
2015 Lineberger Cancer Center Seminar, UNC Chapel Hill, Chapel Hill, N.C.
2015 Annual Meeting of the French Histiocyte Society, Hopital Pitie Salpetriere, Paris, France
2015 Molecular Aspects of Hematology Workshop, Erasmus University, Rotterdam, Netherlands
2015 20th annual meeting of the European Hematology Association (EHA), Invited Speaker
2015 NY Genome Center, 5 Points Seminar Series. New York, N.Y.
2015 Agios Pharmaceuticals, Inc. Cambridge, M.A.
2015 FASEB Hematological Malignancies. Saxton's River, V.T.
2015 National Center for Tumor Disease (NCT)/The German Cancer Research Center (DKFZ) Heidelberg, Germany
2015 European School of Hematology AML Meeting, Budapest, Hungary
2015 U.K. MDS Foundation Forum, London, U.K.
2015 Dept. of Cell and Molecular Biology, SUNY Downstate, Brooklyn, N.Y.
2015 10th Annual CML and MPN Post-ASH Workshop, Orlando, F.L.
2016 Institute for Cancer Genetics Seminar Series, Columbia University
2016 Human Biology Seminar Series, Fred Hutchinson Cancer Research Center, Seattle, WA
2016 5th Annual Symposium of the Critical Reviews in Hematological Malignancies, King Faisal Specialist Hospital and Research Centre, Riyadh, Saudi Arabia
2016 5th International Bone Marrow Failure Disease Scientific Symposium, Aplastic Anemia and MDS International Foundation, Rockville, MD
2016 36th Annual Congress of the French Society of Hematology, Paris, France
2016 Research Seminar, Rigel Pharmaceuticals Inc., South San Francisco, CA
2016 Leukemia Grand Rounds, MD Anderson Cancer Center, Houston, TX
2016 24th Meeting of the Henry Kunkel Society, Rockefeller University, New York, NY
2016 AACR Educational Session, AACR Annual Meeting, New Orleans, LA
2016 AACR Recent Advances in Diagnosis and Therapy Session, AACR Annual Meeting, New Orleans, LA
2016 Research Seminar, Janssen Research & Development, Spring House, PA
2016 Starr Cancer Consortium Retreat, Cold Spring Harbor Laboratories, Cold Spring Harbor, NY
2016 Hematology Grand Rounds, Fred Hutchinson Cancer Research Center, Seattle, WA
2016 Hairy Cell Leukemia Foundation Meeting, Heidelberg, Germany
2016 *Nature Medicine/Nature Biotechnology* SciCafe "Targeting Inhibition of Splicing", New York, NY, USA
2016 1st Dotan International Symposium, Tel Aviv University, Tel Aviv, Israel
2016 4th Annual Erdheim-Chester Disease Medical Symposium, Paris, France
2016 Winthrop Rockefeller Seminar Series, University of Arkansas Medical School, Little Rock, Arkansas, USA
2016 Plenary Speaker, Japanese Society of Hematology, 78th Annual Meeting, Yokohama, Japan
2016 Plenary Speaker, 2nd Spliceosomal Gene Mutations in Cancer Workshop, Broad Institute of Harvard & MIT, Cambridge, MA
2016 Developmental Therapeutics Research Seminar, Amgen Inc., Thousand Oaks, CA
2016 Grand Rounds, Winshop Cancer Institute of Emory University
2016 Ingrim Cancer Center Seminar Series, Vanderbilt University School of Medicine, Nashville, TN
2016 Tisch Cancer Institute Seminar Series, Icahn School of Medicine at Mount Sinai, New York, NY
2016 11th CML & MPN Post-ASH Workshop, La Jolla, California
2016 8th Clinical Translation of Epigenetics and Cancer Therapy, Jekyll Island, GA
2017 Phase Separation and RNA Processing as Drivers of Cancer and Neurodegenerative Disease, UCSD, San Diego, CA
2017 Keystone Symposium RNA Processing in Human Disease, Taos, New Mexico
2017 EHA/ASH Translational Research Training In Hematology Course, Milan, Italy
2017 Curie Institute, Future of Oncology Symposium, Paris, France
2017 Massachusetts General Hospital Cancer Center Seminar Series, Boston, MA

ACTIVE RESEARCH SUPPORT:

National Institutes of Health 7/1/2015 - 6/30/2020
NIH, 1R01 HL128239 (PIs: Bradley / Abdel-Wahab)
"Genetic and molecular basis for SRSF2 mutations in myelodysplasia"

National Institutes of Health 9/1/2016 - 3/31/2020
NIH, 1 R01 CA201247-01A1 (PIs: Abdel-Wahab / Park)
"Origins of BRAF-mutant hematologic malignancies and their therapeutic resistance"

Dept. of Defense, Bone Marrow Failure Research Program 4/1/2016 - 3/31/2018
BM150092 (PI: Abdel-Wahab)
"Therapeutic targeting of spliceosomal mutant acquired bone marrow failure disorders"

Starr Cancer Consortium 1/1/2015 - 12/31/2016
I8-A8-075 (PI: Abdel-Wahab)
"Understanding and Targeting Spliceosomal-Mutation Hematopoietic Malignancies"

Leukemia and Lymphoma Society Scholar Award (PI: Abdel-Wahab) 07/01/2016 - 06/30/2021
"Understanding and targeting Diverse Kinase Alterations in Systemic Histiocytic Neoplasms"

Josie Robertson Investigator (PI: Abdel-Wahab) 9/1/12 – 8/31/17 $
Josie Robertson Investigator Program

Tri-Institutional Stem Cell Initiative (PI: Park) 10/1/14 - 9/30/17
"Hematopoietic stem cell origins of mature B-cell neoplasms"

Starr Cancer Consortium $ 375,000 1/1/2016 - 12/31/2017
I9-A9-059 (PI: Mullally)
"Personalized immunotherapy for the treatment of hematological malignancies"

Hairy Cell Leukemia Foundation (PI: Abdel-Wahab) 1/1/2016 - 12/31/2016
"Functional Characterization of Mutations Collaborating with *BRAF*V600E in HCL"

Erdheim Chester Disease Global Alliance (PI: Abdel Wahab) 1/1/2016 - 12/31/2016
"Identification of novel molecular targets for therapy in Erdheim Chester disease without BRAF mutation"

Histiocytosis Association (PI: Janku) 1/1/2016 - 12/31/2016
"MEK Inhibition in the Therapy of Histiocytic Neoplasms"

Pershing Square Sohn Cancer Research Alliance 7/1/2016 - 6/30/2019
"Identification of novel transcripts, pathways, and therapeutic strategies to target spliceosomal-mutant malignancies"

GC228160 (PI: Abdel-Wahab) 9/1/2016 - 8/31/2017
Edward P. Evans Foundation
"Elucidating Critical Targets, Transcripts, and Collaborating Events in Spliceosomal-Mutant MDS"

PRIOR RESEARCH SUPPORT

When Everyone Survives Award in Leukemia Research (Abdel-Wahab) 7/1/11 – 6/30/12
When Everyone Survives Foundation
"Understanding the biologic and therapeutic relevance of ASXL1 mutations in acute myeloid leukemia"
To determine how exactly mutations in ASXL1 contribute to leukemia development.

LSLF Discovery Research Grant (Abdel-Wahab) 4/1/11 – 3/31/12
Lauri Strauss Leukemia Foundation
"Role of ASXL1 mutations in AML"
The goal of this project is to fully uncover (1) the effect of ASXL1 mutations of outcome in AML, (2) a comprehensive list of the genes whose expression is regulated by ASXL1 and a genome-wide view of the effects of ASXL1 loss on histone proteins at the sites of those genes and (3) the role of ASXL1 loss in the blood cells in a mouse model which we are currently creating.

ASH Scholar Award (Abdel-Wahab) 7/1/11 – 6/30/13
American Society of Hematology
"Role of ASXL1 mutations in myeloid malignancies"
The goal of this project is to investigate the biologic and clinical relevance of ASXL1 mutations and how ASXL1 regulates the epigenetic state of genes involved in normal and malignant hematopoiesis.

Gabrielle's Angel Foundation Fellow Award (Abdel-Wahab) 1/1/11 – 12/31/11
Gabrielle's Angel Foundation (Abdel-Wahab)
"The Role of ASXL1 mutations in leukemia patients"
To identify strategies to aid in the therapy of AML patients with this genetic abnormality.

American Society of Hematology (ASH) Research Training Award for Fellows 7/1/09 – 6/30/10
"Cytokine Signaling in Myeloproliferative Neoplasms."
American Society of Hematology, Research Training Award for Fellows.

Erdheim-Chester Disease Global Alliance Grant 08/01/13 - 07/31/214
Erdheim-Chester Disease Global Alliance
Somatic Genetic Alterations in the Pathogenesis and Therapy of Histiocytic Disorders
Histiocytic disorders, including Langerhans cell histiocytosis (LCH) and Erdheim Chester disease (ECD), represent a wide range of rare diseases with heterogeneous clinical courses and prognoses. The relative rarity and protean clinical nature of the histiocytic disorders has made it difficult to clearly delineate the pathophysiology of these conditions. This grant is aimed at the study of histiocytic disorders using high-throughput unbiased techniques and serially through clinical trials of mutant BRAF inhibition.

Post-doctoral fellowship in Bone Marrow Failure Research 7/1/12 – 6/30/15
DOD, BM110172
Understanding and Targeting Epigenetic Alterations in Acquired Bone Marrow Failure
To understand and target aberrant epigenetic modifiers in the pathogenesis of myelodysplastic syndromes.

American Society of Hematology (PI: Abdel-Wahab) 07/1/14 - 06/30/16
"BRAF Mutations in the Pathogenesis and Therapy of Hairy Cell Leukemia"

Damon Runyon Clinical Investigator Award (PI: Abdel-Wahab) 06/1/13 - 05/30/16
"Understanding and Targeting Altered Histone Modifers in the Myeloid Malignancies"

Clinical Scientist Research Career Development Award (K08) 9/20/11 – 8/31/16
NIH, 1K08CA160647-01 (PI: Abdel-Wahab)
"Role of ASXL1 mutations in myeloid malignancies."

Leukemia and Lymphoma Society (PI: Figueroa) 10/1/2013 - 9/30/2016
"Epigenetic markers of sensitivity and resistance to hypomethylating agents"

The V Foundation (PI: Abdel-Wahab) 10/1/14 - 9/30/16
"Investigating and Targeting Diverse Kinase Alterations Driving

Systemic Histiocytic Neoplasms”

Edward P. Evans Foundation (PI: Abdel-Wahab) 9/1/14 - 8/30/16
“Molecular and Biological Consequences of SRSF2 Mutations in the Myelodysplastic Syndromes”